

**IN THE UNITED STATES DISTRICT COURT  
DISTRICT OF DELAWARE**

NIPPON SHINYAKU CO., LTD.,	)	
	)	
Plaintiff,	)	
	)	
v.	)	C.A. No. 21-1015 (GBW)
	)	
SAREPTA THERAPEUTICS, INC.,	)	
	)	
Defendant.	)	
<hr/>		
SAREPTA THERAPEUTICS, INC. and	)	
THE UNIVERSITY OF WESTERN	)	
AUSTRALIA,	)	
	)	
Defendant/Counter-Plaintiffs,	)	
	)	
v.	)	
	)	
NIPPON SHINYAKU CO., LTD. and	)	
NS PHARMA, INC.,	)	
	)	
Plaintiff/Counter-Defendants.	)	

**APPENDIX TO  
NIPPON SHINYAKU CO. LTD. AND NS PHARMA, INC.'S OBJECTIONS TO  
SPECIAL MASTER ORDER NO. 1 DENYING MOTION TO COMPEL PRODUCTION  
OF LICENSE AGREEMENTS**

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**IN THE UNITED STATES DISTRICT COURT  
FOR THE DISTRICT OF DELAWARE**

NIPPON SHINYAKU, LTD.,

Plaintiff,

v.

SAREPTA THERAPEUTICS, INC.,

Defendant.

C.A. No. 21-1015-GBW

SAREPTA THERAPEUTICS, INC. and  
UNIVERSITY OF WESTERN AUSTRALIA,

Defendant/Counter-Plaintiff,

v.

NIPPON SHINYAKU CO., LTD.  
and NS PHARMA, INC.,

Plaintiff/Counter-Defendants.

**MEMORANDUM OPINION AND SPECIAL MASTER ORDER #2**

Before the Special Master are three discovery disputes between Nippon Shinyaku, Ltd. and NS Pharma, Inc. (collectively, “Nippon Shinyaku”) and Sarepta Therapeutics, Inc. (“Sarepta”) and University of Western Australia (“UWA”). The table below lists the three disputes and the movant for each.

<b>DISPUTES</b>	<b>MOVANT</b>
1. Motion to compel Sarepta to produce license agreements relating to Duchenne Muscular Dystrophy therapies beyond solely exon-skipping therapies and to produce an unredacted version of the Roche Agreement	Nippon Shinyaku
2. Motion to compel Nippon Shinyaku to produce inventor Dr. Takeda for deposition in the United States	Sarepta
3. Motion to compel Sarepta and UWA to produce inventors Drs. Fletcher and McClorey for deposition	Nippon Shinyaku

On June 15, 2023, each party submitted motions for the disputes where it is the movant. D.I. 246, 247. The parties also submitted letter briefing on each of the motions, and the Special Master held a transcribed videoconference hearing on June 21, 2023. D.I. 226. This Memorandum Opinion and Order addresses the parties' motions.

Having considered the letter briefs and arguments presented by the parties, for the reasons set forth below, IT IS HEREBY ORDERED that: (1) Nippon Shinyaku's motion to compel Sarepta to produce license agreements relating to Duchenne Muscular Dystrophy ("DMD") therapies beyond solely exon-skipping therapies and to produce an unredacted version of the Roche Agreement is **DENIED IN PART** and **GRANTED IN PART** (D.I. 247); (2) Sarepta's motion to compel Nippon Shinyaku to produce inventor Dr. Takeda for deposition in the United States is **GRANTED** (D.I. 246); and (3) Nippon Shinyaku's motion to compel Sarepta and UWA to produce inventors Drs. Fletcher and McClorey for deposition is **DENIED** (D.I. 247).

## **I. BACKGROUND**

This case involves the parties' cross-assertions of patent infringement. Nippon Shinyaku asserts infringement of seven patents (the "NS Patents") against Sarepta, and Sarepta and UWA assert infringement of three patents (the "UWA Patents") against Nippon Shinyaku. All of the patents-in-suit relate to exon-skipping antisense oligomer ("ASO") products offered for sale in the United States for the treatment of DMD.

Nippon Shinyaku and National Center of Neurology and Psychiatry ("NCNP") are assignees of the NS patents. The named inventors on the NS patents are Naoki Watanabe, Youhei Satou, Shin'ichi Takeda, and Tetsuya Nagata. Nippon Shinyaku holds the exclusive assertion rights for the NS Patents by way of a license agreement it has with NCNP. NCNP is not a party in this case.

UWA is the assignee of the UWA Patents. The named inventors on the UWA Patents are Stephen Donald Wilton, Sue Fletcher, and Graham McClorey. Sarepta has exclusive rights to the UWA Patents for the treatment of DMD and the right to enforce the UWA Patents.

## **II. LEGAL STANDARD**

Pursuant to Rule 26 of the Federal Rules of Civil Procedure: “[p]arties may obtain discovery regarding any nonprivileged matter that is relevant to any party’s claim or defense and proportional to the needs of the case, considering the importance of the issues at stake in the action, the amount in controversy, the parties’ relative access to relevant information, the parties’ resources, the importance of the discovery in resolving the issues, and whether the burden or expense of the proposed discovery outweighs its likely benefit. Information within this scope of discovery need not be admissible in evidence to be discoverable.” Fed. R. Civ. P. 26(b)(1).

“Generally, a party moving to compel bears the burden of demonstrating the relevance of the requested information.” *Delaware Display Group LLC v. Lenovo Group Ltd.*, No. 13-2018-RGA, 2016 WL 720977, at \*2 (D. Del. Feb. 23, 2016).

## **III. DISCUSSION**

### **A. Nippon Shinyaku’s Motion to Compel Sarepta to Produce License Agreements Relating to DMD therapies Beyond Solely Exon-Skipping Therapies and to Compel Sarepta to Produce an Unredacted Version of the Roche Agreement (Dispute 1)**

Nippon Shinyaku moves to compel Sarepta to produce license agreements relating to all of Sarepta’s AON and DMD therapies beyond solely exon-skipping therapies, and to compel Sarepta to produce an unredacted version of an agreement between Sarepta and Roche (the “Roche Agreement”). D.I. 247; Nippon Shinyaku’s Opening Brief at 1, 2, 4. Nippon Shinyaku argues that license agreements relating to all of Sarepta’s AON and DMD therapies are discoverable and

should be produced because they are relevant to the parties' damages analysis, namely, the reasonable royalty analysis. *Id.* at 2. Nippon Shinyaku argues that the license agreements it seeks are relevant to "[t]he rates paid by the licensee for the use of other patents comparable to the patent in suit" and, therefore should be produced during discovery. *See Georgia-Pacific Corp. v. United States Plywood Corp.*, 318 F. Supp. 112 (S.D.N.Y. 1970) (discussing *Georgia-Pacific* Factor 2). *Id.* at 2.

Nippon Shinyaku also argues that the license agreements for all of Sarepta's AON and DMD therapies are relevant because of "the importance of understanding the relative value of technologies used in the treatment of DMD." *Id.* at 2. According to Nippon Shinyaku, all such documents are relevant to this case and should be produced because the patient populations for Sarepta's various commercial and developmental products and therapies for treating DMD "overlap" and "the relative economic value of each product is inextricably tied to the others." *Id.* at 2.

Regarding the Roche Agreement, Nippon Shinyaku argues that Sarepta should be compelled to produce an unredacted version because the agreement appears to grant Roche "certain rights relating to Vyondys53®" (a Sarepta product accused of infringement in this case) and "other DMD therapies." Nippon Shinyaku's Opening Brief at 3. Although Sarepta has produced a redacted version of the Roche Agreement, Nippon Shinyaku contends that Sarepta's redactions are improper because they impair Nippon Shinyaku's "ability to analyze key aspects of the overall bargain struck between Sarepta and Roche . . . and relevant information regarding current and future DMD products that affect the market for both accused products in this case." *Id.* at 3.

In response, Sarepta argues that Nippon Shinyaku's motion should be denied because it improperly seeks information that is "irrelevant to the claims and defenses of this action" and "goes far beyond the proportionality standard that Rule 26 requires." Sarepta's Opening Brief at

1. Sarepta argues that Nippon's Shinyaku's demand for documents is overbroad and not proportional to the needs of this case because it seeks *all* of Sarepta's agreements and licenses relating to *all* AON and DMD therapies regardless of whether they are exon-skipping therapies. *Id.* at 1. Sarepta also argues that Nippon Shinyaku seeks to obtain sensitive commercial information from Sarepta, a direct competitor, involving different patents and products than those relevant and at issue in this case. *Id.* at 1, 3. Sarepta maintains that it has already produced relevant exon-skipping licenses and agreements commensurate with the scope of the claims and defenses in this case and the parties' prior negotiations on this issue. *Id.* at 3.

Regarding the Roche Agreement, Sarepta argues that Nippon Shinyaku is not entitled to an unredacted version of that agreement because it is not a license. Sarepta's Opening Brief at 3.

[REDACTED]

[REDACTED]

[REDACTED]

Nippon Shinyaku's motion is **DENIED** to the extent that it seeks to compel Sarepta to produce: (1) *all* agreements and licenses relating to *all* DMD therapies, regardless of whether they target exon 53 or are exon-skipping therapies, and (2) *all* of Sarepta's licenses relating to nucleic acid-based therapies known as AONs regardless of whether they skip exons or treat DMD. Nippon Shinyaku's demand for all such documents is overbroad and not proportional to the needs of this case because it is not commensurate with the scope of the claims and defenses in the case and the parties' prior negotiations on this issue. It is undisputed that the patents-in-suit all relate to



exon-skipping ASO products offered for sale in the United States for the treatment of DMD, and specifically to exon-skipping therapies targeting exon 53. Yet, Nippon Shinyaku's motion seeks the production of *all* licenses and agreements for *all* of Sarepta's AON and DMD therapies regardless of whether they target exon 53 or are exon-skipping therapies. It is also undisputed that in prior correspondence with Sarepta regarding this dispute, Nippon Shinyaku confirmed that the scope of the dispute was "[a]ll agreements/licenses related to developing exon-skipping oligonucleotides and/or Vyondys53® . . . ," which is much narrower than the broad scope of discovery Nippon Shinyaku now seeks in its motion.

The Special Master is not persuaded that the broad scope of documents that Nippon Shinyaku seeks is relevant or that all such documents are even potentially comparable to the patents-in-suit. Although Nippon Shinyaku argues that license agreements relating to all of Sarepta's AON and DMD therapies are relevant to the reasonable royalty analysis, i.e., *Georgia-Pacific* factor 2, Nippon Shinyaku does not meaningfully explain how or why all such documents, particularly those unrelated to exon-skipping or DMD treatment therapies, are relevant or otherwise comparable to any of the asserted patents. Nippon Shinyaku's assertions that the requested documents are relevant to "understanding the relative value of technologies used in the treatment of DMD" and that "the relative economic value of each product is inextricably tied to the others" are speculative and, without more, insufficient to satisfy Nippon Shinyaku's burden in this regard.

Nippon Shinyaku's motion is **GRANTED IN PART** to the extent that it seeks to compel Sarepta to produce an unredacted version of the Roche Agreement, subject to the Special Master's *in camera* review of the unredacted Roche Agreement to assess the scope of Sarepta's redactions and facilitate appropriate redactions, as necessary. The Special Master is persuaded, and Sarepta

essentially admits, that at least certain portions of the Roche Agreement are relevant to the accused Vyondys53® product, and therefore, that the Roche Agreement should be produced, at least in part and with appropriate redactions following *in camera* review by the Special Master.

Accordingly, Nippon Shinyaku's motion to compel Sarepta to produce license agreements relating to all of Sarepta's AON and DMD therapies beyond solely exon-skipping therapies, and to compel Sarepta to produce an unredacted version of the Roche Agreement is **DENIED IN PART** and **GRANTED IN PART**.

**IT IS FURTHER HEREBY ORDERED** that within two (2) business days of this Memorandum Opinion and Order, Sarepta shall provide by email only to the Special Master and not to Nippon Shinyaku, a pdf copy of the full, unredacted version of the Roche Agreement, along with a pdf copy of the redacted version of the Roche Agreement that Sarepta has produced in this case. The Special Master will review the documents *in camera* and thereafter provide further guidance regarding scope of Sarepta's redactions.

**B. Sarepta's Motion to Compel Nippon Shinyaku to Produce Inventor Dr. Shin'ichi Takeda for Deposition in the United States (Dispute 2)**

Sarepta moves to compel Nippon Shinyaku to produce Dr. Takeda for deposition in the United States. D.I. 246; Sarepta's Opening Brief at 1. Dr. Takeda is one of the named inventors on the NS Patents and a current employee of NCNP in Japan. *Id.* at 1. Sarepta argues that Nippon Shinyaku should produce Dr. Takeda for deposition in the United States because Dr. Takeda possesses relevant, unique, and discoverable information regarding the claimed subject matter of the NS Patents asserted in this case. *Id.* at 1. Sarepta also argues that Dr. Takeda executed an assignment agreement (the "Assignment Agreement") that granted his interest in the NS Patents

to Nippon Shinyaku and NCNP and obligates him to testify in the United States in patent infringement actions concerning enforcement of the NS Patents. *Id.* at 1.

Sarepta contends that Dr. Takeda's Assignment Agreement, in relevant part, states:

AND I/WE HEREBY further covenant and agree that I/WE will, without further consideration, communicate with assignee . . . any facts known to ME/US respecting this invention and testify in any legal proceeding, . . . and generally do everything possible to aid assignee . . . to obtain and enforce proper patent protection for this invention in the United States . . .

Sarepta's Opening Brief at 1. Sarepta also contends that, in addition to the language in Dr. Takeda's Assignment Agreement, both NCNP and Nippon Shinyaku entered a subsequent litigation agreement that grants Nippon Shinyaku the "exclusive right to pursue infringements of the NS Patents" and requires NCNP to "provide its full cooperation to a reasonable extent, in accordance with the requests by" Nippon Shinyaku. *Id.* at 1.

Sarepta argues that, because courts have found nearly identical assignment agreement language to require the patentee to produce foreign inventors for deposition in the United States, Nippon Shinyaku should be compelled to produce Dr. Takeda for deposition in the United States. *Id.* at 3 (citing *Aerocrine AB v. Apieron Inc.*, 267 F.R.D. 105, 111-12 (D. Del. 2010)).

In response, Nippon Shinyaku argues that Sarepta's motion to compel a deposition of Dr. Takeda in the United States should be denied because Sarepta "fails to give due consideration to the factors governing the proportionality of such a deposition to the needs of this case and fails to provide any justification for why a remote deposition will not be sufficient." Nippon Shinyaku's Response Brief at 1. Nippon Shinyaku argues that Sarepta's demand to depose Dr. Takeda in the United States is not justified because Nippon Shinyaku is already providing four other witnesses for deposition in the United States who it expects to testify about Nippon Shinyaku's research and collaboration with NCNP. *Id.* at 1.

Nippon Shinyaku also argues that Sarepta's demand to depose Dr. Takeda in the United States is disproportionate to the needs of this case because Sarepta offers no argument to justify why an in-person deposition in the United States would be necessary and Nippon Shinyaku is not withholding discovery regarding Dr. Takeda's and NCNP's involvement in the research leading to the NS Patents. *Id.* at 3. Nippon Shinyaku maintains that a deposition of Dr. Takeda would be "highly burdensome, particularly if not located in Japan or conducted remotely." *Id.* at 4.

Sarepta's motion to compel Nippon Shinyaku to produce Dr. Takeda for deposition in the United States is **GRANTED**. In view of the facts of this case, the Special Master is persuaded that the weight of the applicable case law supports Sarepta's position. The Special Master finds the facts and analysis in the *Aerocrine* case that Sarepta cites in its opening letter brief particularly instructive. Similar to the facts in *Aerocrine*, Dr. Takeda is a co-inventor on the asserted NS Patents and possesses knowledge relevant to the claimed subject matter of the asserted patents. Dr. Takeda is also an employee of NCNP and a party to the Assignment Agreement with Nippon Shinyaku and NCNP for the NS Patents that specifically contemplates and expressly requires Dr. Takeda to "testify in any legal proceeding, and generally do everything possible to aid assignee . . . to obtain and enforce proper patent protection for this invention in the United States." *See Aerocrine*, 267 F.R.D. at 111-12 (finding co-inventors similarly obligated under an assignment agreement "to testify in any judicial proceeding . . . and do everything possible to aid [assignee] to obtain and enforce said letter Patent in the United States when requested to do so by the [assignee]"). In addition to the express language in Dr. Takeda's Assignment Agreement, there is no dispute that the subsequent litigation agreement entered between Nippon Shinyaku and NCNP requires NCNP to "provide its full cooperation to a reasonable extent, in accordance with the requests by" Nippon Shinyaku.

Thus, for purposes of providing deposition testimony in this case, the Special Master is persuaded that Nippon Shinyaku has control of Dr. Takeda and is therefore obligated to produce Dr. Takeda for deposition in the United States.

Nippon Shinyaku's arguments that an in-person deposition of Dr. Takeda in the United States is not justified, and that it would be highly burdensome if the deposition is not located in Japan or conducted remotely are not well taken because Nippon Shinyaku has not provided any evidence to support them. Nippon Shinyaku does not, for example, identify any financial hardship or inability by Dr. Takeda to attend a deposition in the United States. Although during the June 21, 2023 Hearing, counsel for Nippon Shinyaku stated that "it is unlikely that [counsel] could get Dr. Takeda and arrange for him to travel to the United States before [August 14, 2023]" and that it was "going to be very difficult, given his schedule," counsel did not state that Dr. Takeda would be unable to attend a deposition in the United States "if the Court orders [counsel] to make him available sooner." *See* June 21, 2023 Hrg. Tr. at 36:23-37:1, 37:16-21.

Accordingly, Sarepta's motion to compel Nippon Shinyaku to produce Dr. Takeda for deposition in the United States is **GRANTED**.

**IT IS FURTHER HEREBY ORDERED** that Nippon Shinyaku shall make Dr. Shin'ichi Takeda available for deposition in the United States at a mutually agreeable date, time, and location within twenty-five (25) days of this Memorandum Opinion and Order.

**C. Nippon Shinyaku's Motion to Compel Sarepta and UWA to Produce Inventors Drs. Fletcher and McClorey for Deposition (Dispute 3)**

Nippon Shinyaku moves to compel Sarepta and UWA to make at least one of Dr. Sue Fletcher and Dr. Graham McClorey available for deposition. D.I. 247; Nippon Shinyaku's Opening Brief at 1. Dr. Fletcher and Dr. McClorey are two of the named co-inventors on the UWA

Patents and both former employees of UWA. *Id.* at 1. They also both reside outside of the United States. *Id.* at 1. Nippon Shinyaku argues that Sarepta and UWA should be compelled to produce Drs. Fletcher and McClorey for deposition in this case “as a matter of fairness and proportionality.”

*Id.* at 5. In particular, Nippon Shinyaku argues that

the Court compel equal numbers of inventor depositions—*i.e.*, [Nippon Shinyaku’s] choice of deposing either Dr. Fletcher or Dr. McClorey should the Court deny Sarepta’s and UWA’s motion regarding [Discovery] Issue #2, and depositions for both Dr. Fletcher and Dr. McClorey should the Court grant that motion.

Nippon Shinyaku’s Opening Brief at 5. Nippon Shinyaku contends it “has a *need* to depose at least one of Dr. Fletcher and/or Dr. McClorey to understand what each of their purported contributions to the claimed exon-53 skipping oligonucleotides were.” *Id.* at 6.

Nippon Shinyaku argues that the Court has authority to order at least UWA to produce Dr. Fletcher and/or Dr. McClorey because Drs. Fletcher and McClorey both assigned their patent rights to the UWA Patents to UWA in an assignment agreement (the “UWA Assignment Agreement”). *Id.* at 7. Nippon Shinyaku contends that the UWA Assignment Agreement, in relevant part, states:

to issue all letters patent on said invention to ASSIGNEE. ASSIGNORS agree to execute all instruments and documents required for the making and prosecution of applications for [REDACTED] on said invention, [REDACTED] or for the purpose of protecting title to said invention or letters patent therefore.

*Id.* at 7. Nippon Shinyaku argues that this language in the UWA Assignment Agreement is akin to language in assignment agreements that courts in the District of Delaware have found to obligate production of foreign inventors for deposition. *Id.* at 7 (citing *Amgen, Inc. v. Ariad Pharms., Inc.*, No. 06-cv-259-MPT, 2007 WL 1425854, at \*2 (D. Del. May 14, 2007) and *Aerocrine AB v. Apieron Inc.*, 267 F.R.D. 105, 111-12 (D. Del. 2010)).

In response, Sarepta and UWA argue that Nippon Shinyaku's motion should be denied because neither Sarepta nor UWA has control over Drs. Fletcher and McClorey to compel them to appear at a deposition. Sarepta's Responsive Brief at 4. Rather, Sarepta and UWA contend that Drs. Fletcher and McClorey are not current employees of either Sarepta or UWA, and that they are both foreign residents: Dr. Fletcher, a resident of Australia and Dr. McClorey, a resident of the U.K. *Id.* at 4. Sarepta and UWA also contend that Drs. Fletcher and McClorey do not have contractual obligations to testify and that the UWA Assignment Agreement they executed transferring patent rights to UWA does not even mention testifying. *Id.* at 4. Instead, Sarepta and UWA maintain that the UWA Assignment Agreement only obligates Drs. Fletcher and McClorey to sign documents. *Id.* at 4.

Nippon Shinyaku's motion to compel Sarepta and UWA to produce inventors Drs. Fletcher and McClorey for deposition is **DENIED**. Neither Sarepta nor UWA has control of Drs. Fletcher and McClorey to compel them to appear at a deposition. Drs. Fletcher and McClorey are also not current employees of Sarepta or UWA, and the UWA Assignment Agreement they executed does not contain any specific language obligating them to testify in any legal proceeding or appear for a deposition. Rather, as Sarepta and UWA correctly point out, all that the UWA Assignment Agreement obligates Drs. Fletcher and McClorey to do is sign documents. The UWA Assignment Agreement merely states that "ASSIGNORS agree to execute all instruments and documents required . . . for litigation regarding letters patent, or for the purpose of protecting title to said invention or letters patent therefore." Sarepta's Responsive Brief at 5.

Nippon Shinyaku's reliance on the *Aerocrine* case is misplaced. In contrast to the assignment agreement in *Aerocrine* and Dr. Takeda's Assignment Agreement discussed above in connection with Dispute 2, the UWA Assignment Agreement does not include specific language

that requires Drs. Fletcher and McClorey “*to testify in any legal proceeding*” regarding the UWA Patents. *See Aerocrine*, 267 F.R.D. at 111-12 (emphasis added).

Nippon Shinyaku’s reliance on the *Amgen* case is also misplaced. In contrast to the UWA Assignment Agreement, the assignment agreement in *Amgen* required inventors “to perform any other lawful acts which may be deemed necessary to secure fully the aforesaid invention,” including “*the giving of testimony in any interference or other proceeding* in which said invention or . . . or patent directed thereto may be involved.” *Amgen*, 2007 WL 1425854, at \*1 (emphasis added).

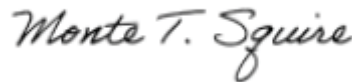
Accordingly, Nippon Shinyaku’s motion to compel Sarepta and UWA to make at least one of Dr. Sue Fletcher and Dr. Graham McClorey available for deposition is **DENIED**.

\* \* \*

This Memorandum Opinion and Order is preliminarily submitted under seal as a precaution because various portions of the underlying briefing and June 21, 2023 Hearing Transcript were marked highly confidential. Within three (3) business days of this Order, the parties shall jointly email the Special Master and advise of any proposed redactions.

IT IS SO ORDERED.

Dated: July 6, 2023



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Special Master Monté T. Squire





## Sarepta Therapeutics Announces FDA Approval of ELEVIDYS, the First Gene Therapy to Treat Duchenne Muscular Dystrophy

6/22/23

- ***ELEVIDYS (delandistrogene moxeparvovec-rokl) is approved for the treatment of ambulatory pediatric patients aged 4 through 5 years with Duchenne based on expression of ELEVIDYS micro-dystrophin observed in patients treated with ELEVIDYS***
- ***ELEVIDYS is a one-time treatment designed to treat the underlying genetic cause of Duchenne***
- ***Sarepta will host a conference call on June 22 at 4:30 p.m. ET***

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jun. 22, 2023-- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), the leader in precision genetic medicine for rare diseases, today announced U.S. Food and Drug Administration (FDA) accelerated approval of ELEVIDYS (delandistrogene moxeparvovec-rokl), an adeno-associated virus based gene therapy for the treatment of ambulatory pediatric patients aged 4 through 5 years with Duchenne muscular dystrophy (DMD) with a confirmed mutation in the *DMD* gene. This indication is approved under accelerated approval based on expression of ELEVIDYS micro-dystrophin observed in patients treated with ELEVIDYS. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trial(s). ELEVIDYS is contraindicated in patients with any deletion in exon 8 and/or exon 9 in the *DMD* gene.

This press release features multimedia. View the full release here: <https://www.businesswire.com/news/home/20230622454844/en/>

# Elevidys

## delandistrogene moxeparvovec-rokl

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### suspension for intravenous infusion

ELEVIDYS addresses the root genetic cause of Duchenne – mutations in the dystrophin gene that result in the lack of dystrophin protein – by delivering a gene that codes for a shortened form of dystrophin to muscle cells known as ELEVIDYS micro-dystrophin. This accelerated approval is based on an increase in ELEVIDYS micro-dystrophin protein expression in skeletal muscle. ELEVIDYS is supported by biologic and empirical evidence, in addition to efficacy data from two clinical studies: SRP-9001-102 and SRP-9001-103 and safety data from SRP-9001-101, SRP-9001-102 and SRP-9001-103. Acute serious liver injury, immune-mediated myositis and myocarditis have occurred in patients treated with ELEVIDYS. The most common adverse reactions in clinical studies were vomiting, nausea, liver function test increased, pyrexia and thrombocytopenia.

ELEVIDYS is the first FDA approved gene therapy to treat Duchenne muscular dystrophy. (Graphic: Business Wire)

Consistent with the accelerated approval pathway, the company has committed to the completion of a confirmatory trial. EMBARK, the global, randomized, double-

blind, placebo-controlled Phase 3 trial for ELEVIDYS, will serve as the post-marketing confirmatory trial and is fully enrolled with top-line results expected in late 2023.

"Duchenne is a relentlessly progressive, degenerative disease, robbing children of muscle function<sup>1</sup>," said Jerry Mendell, M.D., pediatric neurologist and principal investigator in the Center for Gene Therapy at Nationwide Children's Hospital. "The increases in ELEVIDYS dystrophin expression and the functional results that we see can make a difference in the lives of our patients."

"The approval of ELEVIDYS is a watershed moment for the treatment of Duchenne. ELEVIDYS is the first and only gene therapy approved for Duchenne, and this approval brings us closer to our goal of bringing forward a treatment that provides the potential to alter the trajectory of this degenerative disease," said Doug Ingram, president and chief executive officer, Sarepta. "As we prepare to launch ELEVIDYS, we should acknowledge and celebrate the decades of dedication and work from the patient community, families, clinicians, and our Sarepta colleagues that resulted in today's approval. Our confirmatory trial, EMBARK, should read out in the fourth quarter of this year. If EMBARK confirms the benefits seen in our prior trials, Sarepta will move rapidly to submit a BLA supplement to expand the approved label as broadly as good science permits."

"Today's decision marks an important moment in gene therapy for patients living with Duchenne," said Pat Furlong, founding president and chief executive officer, Parent Project Muscular Dystrophy. "It's been the lifelong work of so many in the Duchenne community. Our work continues until all

patients in our community have access to therapy."

Patients and physicians can access more information at [www.SareptAssist.com](http://www.SareptAssist.com) or by calling 1-888-727-3782.

#### **Conference call details**

At 4:30 p.m. June 22, 2023, Sarepta will host a conference call and webcast to discuss this update.

The event will be webcast live under the investor relations section of Sarepta's website at <https://investorrelations.sarepta.com/events-presentations> and following the event a replay will be archived there for one year. Interested parties participating by phone will need to register using [this online form](#). After registering for dial-in details, all phone participants will receive an auto-generated e-mail containing a link to the dial-in number along with a personal PIN number to use to access the event by phone.

#### **About ELEVIDYS (delandistrogene moxeparvec-rokl)**

ELEVIDYS (delandistrogene moxeparvec-rokl) is a single-dose gene transfer therapy for intravenous infusion designed to address the underlying cause of Duchenne muscular dystrophy through the targeted production of ELEVIDYS micro-dystrophin in skeletal muscle. ELEVIDYS has been evaluated in three on-going clinical studies: SRP-9001-101, SRP-9001-102 and SRP-9001-103. Accelerated approval was primarily based on data from SRP-9001-102 and SRP-9001-103. More than 80 treated patients across the three studies contributed to the safety profile of ELEVIDYS. ELEVIDYS is also being studied in Study SRP-9001-301 (also known as EMBARK), a global, randomized, double-blind, placebo-controlled Phase 3 clinical trial in 126 participants with Duchenne between the ages of 4 to 7 years.

#### **IMPORTANT SAFETY INFORMATION**

##### **CONTRAINDICATION:**

ELEVIDYS is contraindicated in patients with any deletion in exon 8 and/or exon 9 in the *DMD* gene.

##### **WARNINGS AND PRECAUTIONS:**

###### **Acute Serious Liver Injury:**

- Acute serious liver injury has been observed with ELEVIDYS. Administration of ELEVIDYS may result in elevations of liver enzymes (e.g., GGT, GLDH, ALT, AST) or total bilirubin, typically seen within 8 weeks.
- Patients with preexisting liver impairment, chronic hepatic condition, or acute liver disease (e.g., acute hepatic viral infection) may be at higher risk of acute serious liver injury. Postpone ELEVIDYS administration in patients with acute liver disease until resolved or controlled.
- Prior to ELEVIDYS administration, perform liver enzyme test and monitor liver function (clinical exam, GGT, and total bilirubin) weekly for the first 3 months following ELEVIDYS infusion. Continue monitoring if clinically indicated, until results are unremarkable (normal clinical exam, GGT and total bilirubin levels return to near baseline levels).
- Systemic corticosteroid treatment is recommended for patients before and after ELEVIDYS infusion. Adjust corticosteroid regimen when indicated. If acute serious liver injury is suspected, a consultation with a specialist is recommended.

###### **Immune-mediated Myositis:**

- In clinical trials, immune-mediated myositis has been observed approximately 1 month following ELEVIDYS infusion in patients with deletion mutations involving exon 8 and/or exon 9 in the *DMD* gene. Symptoms of severe muscle weakness including dysphagia, dyspnea and hypophonia were observed.
- Limited data are available for ELEVIDYS treatment in patients with mutations in the *DMD* gene between exons 1 to 17 and exons 59 to 71. Patients with deletions in these regions may be at risk for a severe immune-mediated myositis reaction.
- Advise patients to contact a physician immediately if they experience any unexplained increased muscle pain, tenderness, or weakness, including dysphagia, dyspnea or hypophonia as these may be symptoms of myositis. Consider additional immunomodulatory treatment (immunosuppressants [e.g., calcineurin-inhibitor] in addition to corticosteroids) based on patient's clinical presentation and medical history if these symptoms occur.

###### **Myocarditis:**

- Acute serious myocarditis and troponin-I elevations have been observed following ELEVIDYS infusion in clinical trials.
- Monitor troponin-I before ELEVIDYS infusion and weekly for the first month following infusion and continue monitoring if clinically indicated. More frequent monitoring may be warranted in the presence of cardiac symptoms, such as chest pain or shortness of breath.
- Advise patients to contact a physician immediately if they experience cardiac symptoms.

###### **Pre-existing Immunity against AAVrh74:**

- In AAV-vector based gene therapies, preexisting anti-AAV antibodies may impede transgene expression at desired therapeutic levels. Following treatment with ELEVIDYS, all subjects developed anti-AAVrh74 antibodies.
- Perform baseline testing for the presence of anti-AAVrh74 total binding antibodies prior to ELEVIDYS administration.
- ELEVIDYS administration is not recommended in patients with elevated anti-AAVrh74 total binding antibody titers greater

than or equal to 1:400.

#### **Adverse Reactions:**

- The most common adverse reactions (incidence  $\geq 5\%$ ) reported in clinical studies were vomiting, nausea, liver function test increased, pyrexia, and thrombocytopenia.

Sarepta is responsible for global development and manufacturing for ELEVIDYS, and distribution within the U.S. will commence immediately. In December 2019, Sarepta partnered with Roche to accelerate access to ELEVIDYS for patients outside the United States.

ELEVIDYS is approved under accelerated review based on expression of ELEVIDYS micro-dystrophin in skeletal muscle. Continued approval for this indication in this and other age groups will be contingent upon verification of a clinical benefit in confirmatory trials. ELEVIDYS has met the full statutory standards for safety and effectiveness and as such is not considered investigational or experimental.

For further information, please see the full [Prescribing Information](#).

#### **About Sarepta Therapeutics**

Sarepta is on an urgent mission: engineer precision genetic medicine for rare diseases that devastate lives and cut futures short. We hold leadership positions in Duchenne muscular dystrophy (DMD) and limb-girdle muscular dystrophies (LGMDs), and we currently have more than 40 programs in various stages of development. Our vast pipeline is driven by our multi-platform Precision Genetic Medicine Engine in gene therapy, RNA and gene editing. For more information, please visit [www.sarepta.com](http://www.sarepta.com) or follow us on [Twitter](#), [LinkedIn](#), [Instagram](#) and [Facebook](#).

#### **Internet Posting of Information**

We routinely post information that may be important to investors in the 'For Investors' section of our website at [www.sarepta.com](http://www.sarepta.com). We encourage investors and potential investors to consult our website regularly for important information about us.

#### **Forward-Looking Statements**

*This press release contains "forward-looking statements." Any statements that are not statements of historical fact may be deemed to be forward-looking statements. Words such as "believe," "anticipate," "plan," "expect," "will," "may," "intend," "prepare," "look," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include, without limitation, statements relating to our future operations, business plans, priorities, research and development programs; ELEVIDYS' continued approval potentially being contingent upon verification and description of clinical benefit in confirmatory trial(s); the potential for ELEVIDYS to bring us closer to our goal of bringing forward a treatment that provides the potential to alter the trajectory of degenerative disease; the potential benefits and risks of ELEVIDYS; and expected plans and milestones, including our expectation of EMBARK serving as the post-marketing confirmatory trial, rapidly moving to submit a BLA supplement to expand the approved label as broadly as good science permits if EMBARK confirms the benefits seen in prior trials, and receiving top-line results from EMBARK in late 2023.*

*Actual results could materially differ from those stated or implied by these forward-looking statements as a result of such risks and uncertainties. Known risk factors include the following: the FDA may not approve a supplement to expand the approved label for ELEVIDYS; continued approval may be contingent upon verification of a clinical benefit in confirmatory trials; we may not be able to comply with all FDA requests in a timely manner or at all; the possible impact of regulations and regulatory decisions by the FDA and other regulatory agencies on our business, as well as the development of our product candidates and our financial and contractual obligations; our dependence on certain manufacturers to produce our products and product candidates, including any inability on our part to accurately anticipate product demand and to secure in a timely manner manufacturing capacity to meet product demand, may impair the availability of product to successfully support various programs; our data may not be sufficient for obtaining regulatory approval; we are subject to uncertainty related to reimbursement policies; success in preclinical and clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful, and the results of future research may not be consistent with past positive results or with advisory committee recommendations, or may fail to meet regulatory approval requirements for the safety and efficacy of product candidates; the commencement and completion of our clinical trials and announcement of results may be delayed or prevented for a number of reasons, including, among others, denial by the regulatory agencies of permission to proceed with our clinical trials, or placement of a clinical trial on hold, challenges in identifying, recruiting, enrolling and retaining patients to participate in clinical trials and inadequate quantity or quality of supplies of a product candidate or other materials necessary to conduct clinical trials; different methodologies, assumptions and applications we use to assess particular safety or efficacy parameters may yield different statistical results, and even if we believe the data collected from clinical trials of our product candidates are positive, these data may not be sufficient to support approval by the FDA or other global regulatory authorities; we may not be able to execute on our business plans, including meeting our expected or planned regulatory milestones and timelines, research and clinical development plans, and bringing our product candidates to market, for various reasons, many of which may be outside of our control, including possible limitations of company financial and other resources, manufacturing limitations that may not be anticipated or resolved for in a timely manner, regulatory, court or agency decisions, such as decisions by the United States Patent and Trademark Office with respect to patents that cover our product candidates; and those risks identified under the heading "Risk Factors" in our most recent Annual Report on Form 10-K for the year ended December 31, 2022, and Form 10-Q filed with the Securities and Exchange Commission (SEC) as well as other SEC filings made by the Company, which you are encouraged to review.*

*Any of the foregoing risks could materially and adversely affect the Company's business, results of operations and the trading price of Sarepta's common stock. For a detailed description of risks and uncertainties Sarepta faces, you are encouraged to review the SEC filings made by Sarepta. We caution investors not to place considerable reliance on the forward-looking statements contained in this press release. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof, except as required by law.*

<sup>i</sup> Duan D, et al. Nat Rev Dis Primers. 2021;7(1):13.

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Source: Sarepta Therapeutics, Inc.



# Building an industry-leading genetic medicine pipeline

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Sarepta’s industry leading pipeline is comprised of over 40 programs in various stages of development across 3 technologies, RNA, gene therapy and gene editing, and multiple therapeutic areas including neuromuscular, CNS and cardiology. Currently, Sarepta has one gene therapy and three RNA-based therapies on the market in the United States.

## Learn about our Products

### Program Details

*Information is current as of 6/23/2023, updates are made on a quarterly basis*

Program Name	Discovery/Preclinical	Clinical
<b>RNA Targeted Therapies PPMO<sup>1</sup></b>		
SRP-5051 (vesleteplirsen)	Duchenne	
Other Exon Targets <sup>2</sup>	Duchenne	
<hr/>		
<b>Gene Therapy</b>		
GALGT2 - Nationwide Children's	Duchenne	

GNT 0004 - Genethon	Duchenne
SRP-9003 (bidridistrogene xeboparvovec)	LGMD2E/R4 $\beta$ -sarcoglycan
SRP-9004 (patidistrogene bexoparvovec)	LGMD2D/R3 $\alpha$ -sarcoglycan
SRP-6004	LGMD2B/R2 Dysferlin
Other LGMD Targets <sup>3</sup>	LGMD
Other Targets	Multiple

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## Gene Editing

CRISPR/CAS9 - Duke University	Duchenne
CRISPR/CAS9 - Harvard University	Duchenne

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<sup>1</sup>Peptide phosphorodiamidate morpholino oligomers

<sup>2</sup>Other exon targets in development: 44, 45, 50, 52, and 53

<sup>3</sup>Other LGMD targets in development: SRP-9005 (LGMD2C/R5  $\gamma$ -sarcoglycan), SRP-9006 (LGMD2L/R12 Anoctamin 5), and Calpain 3 (LGMD2A/R1)

## Download PDF of Our Pipeline

For more information about our pipeline, please contact Sarepta's Patient Affairs team at [Advocacy@Sarepta.com](mailto:Advocacy@Sarepta.com).

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# Providing FDA-approved therapies

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- [Vyondys 53 website for healthcare professionals](#)



- [U.S. Full Prescribing Information \(PDF\)](#)
- [Amondys 45 website](#)



# SareptAssist

SareptAssist is a patient support program designed to offer information to help you navigate the process of starting and staying on therapy.

**Call 1-888-SAREPTA (1-888-727-3782)**

Available Monday through Friday, 8:30am - 6:30pm ET.  
Spanish-speaking Case Managers and interpreters for other languages are available.

**Visit [SareptAssist.com](https://www.sarepta.com) to  
Get Started**

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**HIGHLIGHTS OF PRESCRIBING INFORMATION**

These highlights do not include all the information needed to use VYONDYS 53 safely and effectively. See full prescribing information for VYONDYS 53.

**VYONDYS 53 (golodirsen) injection, for intravenous use**  
Initial U.S. Approval: 2019

**RECENT MAJOR CHANGES**

Dosage and Administration (2.1, 2.2, 2.3, 2.4)	2/2021
Warnings and Precautions (5.2)	2/2021

**INDICATIONS AND USAGE**

VYONDYS 53 is an antisense oligonucleotide indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the *DMD* gene that is amenable to exon 53 skipping. This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with VYONDYS 53. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials. (1)

**DOSAGE AND ADMINISTRATION**

- Serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio should be measured before starting VYONDYS 53 (2.1)
- 30 milligrams per kilogram once weekly (2.2)
- Administer as an intravenous infusion over 35 to 60 minutes via an in-line 0.2 micron filter (2.2, 2.4)
- Dilution required prior to administration (2.3)

**DOSAGE FORMS AND STRENGTHS**

Injection: 100 mg/2 mL (50 mg/mL) in a single-dose vial (3)

**CONTRAINDICATIONS**

None (4)

**WARNINGS AND PRECAUTIONS**

- **Hypersensitivity Reactions:** Hypersensitivity reactions, including rash, pyrexia, pruritus, urticaria, dermatitis, and skin exfoliation have occurred in patients who were treated with VYONDYS 53. If a hypersensitivity reaction occurs, institute appropriate medical treatment and consider slowing the infusion or interrupting the VYONDYS 53 therapy. (2.3, 5.1)
- **Kidney Toxicity:** Based on animal data, may cause kidney toxicity. Kidney function should be monitored; creatinine may not be a reliable measure of renal function in DMD patients. (5.2, 13.2)

**ADVERSE REACTIONS**

The most common adverse reactions (incidence  $\geq 20\%$  and higher than placebo) were headache, pyrexia, fall, abdominal pain, nasopharyngitis, cough, vomiting, and nausea. (6.1)

To report SUSPECTED ADVERSE REACTIONS, contact Sarepta Therapeutics, Inc. at 1-888-SAREPTA (1-888-727-3782) or FDA at 1-800-FDA-1088 or [www.fda.gov/medwatch](http://www.fda.gov/medwatch).

See 17 for PATIENT COUNSELING INFORMATION

Revised: 2/2021

**FULL PRESCRIBING INFORMATION: CONTENTS\***

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\*Sections or subsections omitted from the full prescribing information are not listed.

## FULL PRESCRIBING INFORMATION

### 1 INDICATIONS AND USAGE

VYONDYS 53 is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the *DMD* gene that is amenable to exon 53 skipping. This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with VYONDYS 53 [see *Clinical Studies* (14)]. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

### 2 DOSAGE AND ADMINISTRATION

#### 2.1 Monitoring to Assess Safety

Serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio should be measured before starting VYONDYS 53. Consider measurement of glomerular filtration rate prior to initiation of VYONDYS 53. Monitoring for kidney toxicity during treatment is recommended. Obtain the urine samples prior to infusion of VYONDYS 53 or at least 48 hours after the most recent infusion [see *Warnings and Precautions* (5.2)].

#### 2.2 Dosing Information

The recommended dosage of VYONDYS 53 is 30 milligrams per kilogram administered once weekly as a 35 to 60-minute intravenous infusion via an in-line 0.2 micron filter.

If a dose of VYONDYS 53 is missed, it may be administered as soon as possible after the scheduled dose.

#### 2.3 Preparation Instructions

VYONDYS 53 is supplied in single-dose vials as a preservative-free concentrated solution that requires dilution prior to administration. Parenteral drug products should be inspected visually for particulate matter and discoloration prior to administration, whenever solution and container permit. Use aseptic technique.

- a. Calculate the total dose of VYONDYS 53 to be administered based on the patient's weight and the recommended dose of 30 milligrams per kilogram. Determine the volume of VYONDYS 53 needed and the correct number of vials to supply the full calculated dose.
- b. Allow the vials to warm to room temperature. Mix the contents of each vial by gently inverting 2 or 3 times. Do not shake.
- c. Visually inspect each vial of VYONDYS 53. The solution is a clear to slightly opalescent, colorless liquid, and may contain trace amounts of small, white to off-white amorphous particles. Do not use if the solution in the vials is cloudy, discolored or

contains extraneous particulate matter other than trace amounts of small, white to off-white amorphous particles.

- d. With a syringe fitted with a 21-gauge or smaller bore non-coring needle, withdraw the calculated volume of VYONDYS 53 from the appropriate number of vials.
- e. Dilute the withdrawn VYONDYS 53 in 0.9% Sodium Chloride Injection, USP, to make a total volume of 100 to 150 mL. Gently invert 2 to 3 times to mix. Do not shake. Visually inspect the diluted solution. Do not use if the solution is cloudy, discolored or contains extraneous particulate matter other than trace amounts of small, white to off-white amorphous particles.
- f. Administer the diluted solution via an in-line 0.2 micron filter.
- g. VYONDYS 53 contains no preservatives and should be administered immediately after dilution. Complete infusion of diluted VYONDYS 53 within 4 hours of dilution. If immediate use is not possible, the diluted product may be stored for up to 24 hours at 2°C to 8°C (36°F to 46°F). Do not freeze. Discard unused VYONDYS 53.

## 2.4 Administration Instructions

Application of a topical anesthetic cream to the infusion site prior to administration of VYONDYS 53 may be considered.

VYONDYS 53 is administered via intravenous infusion. Flush the intravenous access line with 0.9% Sodium Chloride Injection, USP, prior to and after infusion.

Infuse the diluted VYONDYS 53 over 35 to 60 minutes via an in-line 0.2 micron filter. Do not mix other medications with VYONDYS 53 or infuse other medications concomitantly via the same intravenous access line with VYONDYS 53.

If a hypersensitivity reaction occurs, consider slowing the infusion or interrupting the VYONDYS 53 therapy [see *Warnings and Precautions (5.1)* and *Adverse Reactions (6.1)*].

## 3 DOSAGE FORMS AND STRENGTHS

VYONDYS 53 is a clear to slightly opalescent, colorless liquid, and may contain trace amounts of small, white to off-white amorphous particles, and available as:

- Injection: 100 mg/2 mL (50 mg/mL) solution in a single-dose vial

## 4 CONTRAINDICATIONS

None.

## **5 WARNINGS AND PRECAUTIONS**

### **5.1 Hypersensitivity Reactions**

Hypersensitivity reactions, including rash, pyrexia, pruritus, urticaria, dermatitis, and skin exfoliation have occurred in VYONDYS 53-treated patients, some requiring treatment. If a hypersensitivity reaction occurs, institute appropriate medical treatment and consider slowing the infusion or interrupting the VYONDYS 53 therapy [see *Dosage and Administration* (2.4)].

### **5.2 Kidney Toxicity**

Kidney toxicity was observed in animals who received golodirsen [see *Use in Specific Populations* (8.4)]. Although kidney toxicity was not observed in the clinical studies with VYONDYS 53, the clinical experience with VYONDYS 53 is limited, and kidney toxicity, including potentially fatal glomerulonephritis, has been observed after administration of some antisense oligonucleotides. Kidney function should be monitored in patients taking VYONDYS 53. Because of the effect of reduced skeletal muscle mass on creatinine measurements, creatinine may not be a reliable measure of kidney function in DMD patients. Serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio should be measured before starting VYONDYS 53. Consider also measuring glomerular filtration rate using an exogenous filtration marker before starting VYONDYS 53. During treatment, monitor urine dipstick every month, and serum cystatin C and urine protein-to-creatinine ratio every three months. Only urine expected to be free of excreted VYONDYS 53 should be used for monitoring of urine protein. Urine obtained on the day of VYONDYS 53 infusion prior to the infusion, or urine obtained at least 48 hours after the most recent infusion, may be used. Alternatively, use a laboratory test that does not use the reagent pyrogallol red, as this reagent has the potential to cross react with any VYONDYS 53 that is excreted in the urine and thus lead to a false positive result for urine protein.

If a persistent increase in serum cystatin C or proteinuria is detected, refer to a pediatric nephrologist for further evaluation.

## **6 ADVERSE REACTIONS**

The following serious adverse reactions are described below and elsewhere in the labeling:

- Hypersensitivity Reactions [see *Warnings and Precautions* (5.1)]

### **6.1 Clinical Trials Experience**

Because clinical trials are conducted under widely varying conditions, adverse reaction rates observed in the clinical trials of a drug cannot be directly compared to rates in the clinical trials of another drug and may not reflect the rates observed in practice.

In the VYONDYS 53 clinical development program, 58 patients received at least one intravenous dose of VYONDYS 53, ranging between 4 mg/kg (0.13 times the recommended dosage) and 30 mg/kg (the recommended dosage). All patients were male and had genetically confirmed Duchenne muscular dystrophy. Age at study entry was 6 to 13 years. Most (86%) patients were Caucasian.

VYONDYS 53 was studied in 2 double-blind, placebo-controlled studies.

In Study 1 Part 1, patients were randomized to receive once-weekly intravenous infusions of VYONDYS 53 (n=8) in four increasing dose levels from 4 mg/kg to 30 mg/kg or placebo (n=4), for at least 2 weeks at each level. All patients who participated in Study 1 Part 1 (n=12) were continued into Study 1 Part 2, an open-label extension, during which they received VYONDYS 53 at a dose of 30 mg/kg IV once weekly [see *Clinical Studies (14)*].

In Study 2, patients received VYONDYS 53 (n=33) 30 mg/kg or placebo (n=17) IV once weekly for up to 96 weeks, after which all patients received VYONDYS 53 at a dose of 30 mg/kg.

Adverse reactions observed in at least 20% of treated patients in the placebo-controlled sections of Studies 1 and 2 are shown in [Table 1](#).

**Table 1: Adverse Reactions That Occurred in At Least 20% of VYONDYS 53-Treated Patients and at a Rate Greater than Placebo in Studies 1 and 2**

Adverse Reaction	VYONDYS 53 (N = 41) %	Placebo (N = 21) %
Headache	41	10
Pyrexia	41	14
Fall	29	19
Abdominal pain	27	10
Nasopharyngitis	27	14
Cough	27	19
Vomiting	27	19
Nausea	20	10

Other adverse reactions that occurred at a frequency greater than 5% of VYONDYS 53-treated patients and at a greater frequency than placebo were: administration site pain, back pain, pain, diarrhea, dizziness, ligament sprain, contusion, influenza, oropharyngeal pain, rhinitis, skin abrasion, ear infection, seasonal allergy, tachycardia, catheter site related reaction, constipation, and fracture.

Hypersensitivity reactions have occurred in patients treated with VYONDYS 53 [see *Warnings and Precautions (5.1)*].

## **8 USE IN SPECIFIC POPULATIONS**

### **8.1 Pregnancy**

#### Risk Summary

There are no human or animal data available to assess the use of VYONDYS 53 during pregnancy. In the U.S. general population, major birth defects occur in 2 to 4% and miscarriage occurs in 15 to 20% of clinically recognized pregnancies.

### **8.2 Lactation**

#### Risk Summary

There are no human or animal data to assess the effect of VYONDYS 53 on milk production, the presence of golodirsén in milk, or the effects of VYONDYS 53 on the breastfed infant.

The developmental and health benefits of breastfeeding should be considered along with the mother's clinical need for VYONDYS 53 and any potential adverse effects on the breastfed infant from VYONDYS 53 or from the underlying maternal condition.

### **8.4 Pediatric Use**

VYONDYS 53 is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the *DMD* gene that is amenable to exon 53 skipping, including pediatric patients [*see Clinical Studies (14)*].

Intravenous administration of golodirsén (0, 100, 300, or 900 mg/kg) to juvenile male rats once weekly for 10 weeks (postnatal days 14 to 77) did not result in postnatal developmental (e.g., neurobehavioral, immune function, or male reproductive) toxicity. However, at the highest dose tested (900 mg/kg/week), golodirsén resulted in the death of animals because of renal impairment or failure. In surviving animals (including one animal at the lowest dose tested), there was a dose-dependent increase in the incidence and severity of renal tubular effects (including degeneration/regeneration, fibrosis, vacuolation, and dilatation), which correlated with changes in clinical pathology parameters, reflecting a dose-dependent impairment of renal function. In addition, decreases in bone area, mineral content, and mineral density were observed at the highest dose tested (900 mg/kg/week) but with no effect on bone growth. A no-effect dose for renal toxicity was not identified; the lowest dose tested (100 mg/kg/week) was associated with plasma exposures (AUC) approximately 2.5 times that in humans at the recommended human dose of 30 mg/kg/week.

### **8.5 Geriatric Use**

DMD is largely a disease of children and young adults; therefore, there is no geriatric experience with VYONDYS 53.



## 8.6 Patients with Renal Impairment

Renal clearance of golodirsen is reduced in non-DMD adults with renal impairment, based on estimated glomerular filtration rate calculated using the Modification of Diet and Renal Disease (MDRD) equation [see *Clinical Pharmacology* (12.3)]. However, because of the effect of reduced skeletal muscle mass on creatinine measurements in DMD patients, no specific dosage adjustment can be recommended for DMD patients with renal impairment based on estimated glomerular filtration rate. Patients with known renal function impairment should be closely monitored during treatment with VYONDYS 53.

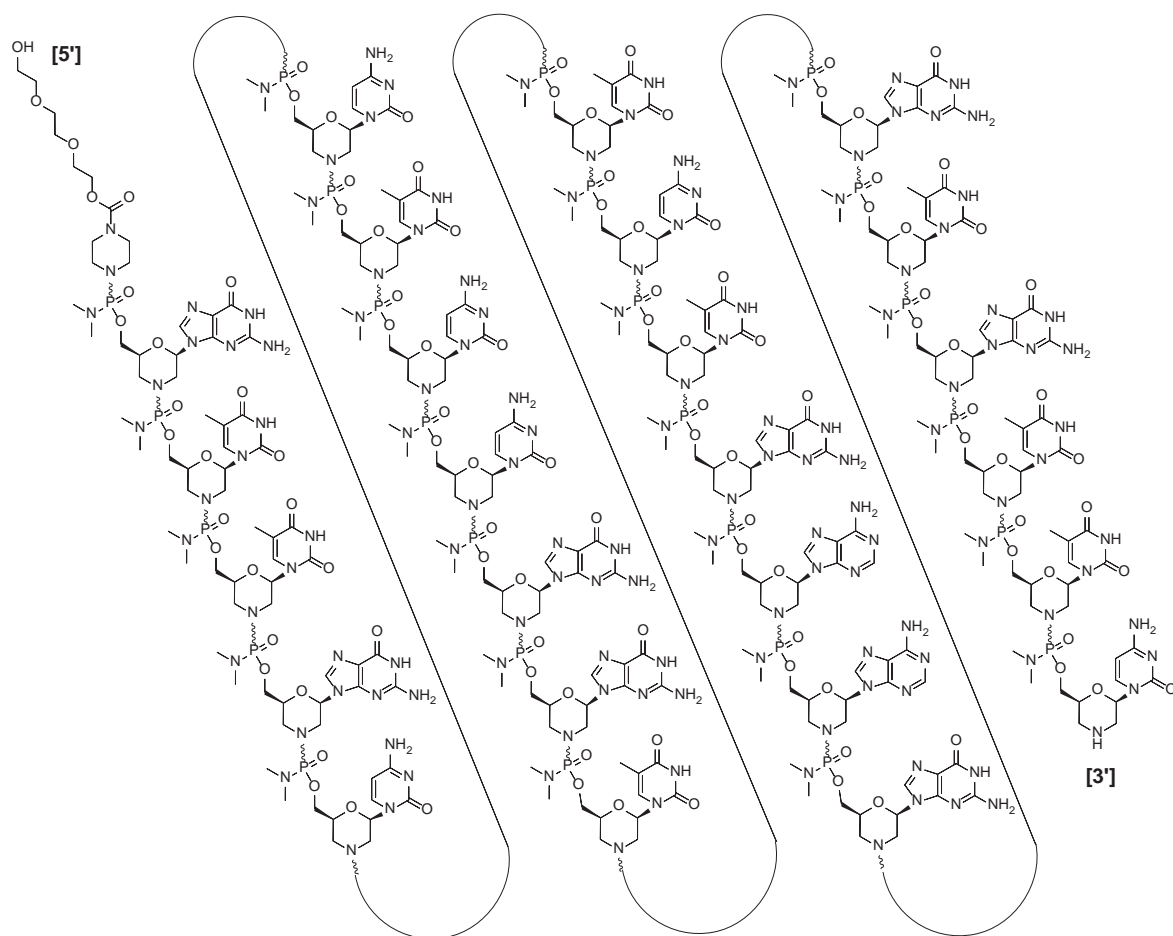
## 11 DESCRIPTION

VYONDYS 53 (golodirsen) injection is a sterile, aqueous, preservative-free, concentrated solution for dilution prior to intravenous administration. VYONDYS 53 is a clear to slightly opalescent, colorless liquid, and may contain trace amounts of small, white to off-white amorphous particles. VYONDYS 53 is supplied in single-dose vials containing 100 mg golodirsen (50 mg/mL). VYONDYS 53 is formulated as an isotonic phosphate buffered saline solution with an osmolality of 260 to 320 mOSM and a pH of 7.5. Each milliliter of VYONDYS 53 contains: 50 mg golodirsen; 0.2 mg potassium chloride; 0.2 mg potassium phosphate monobasic; 8 mg sodium chloride; and 1.14 mg sodium phosphate dibasic, anhydrous, in water for injection. The product may contain hydrochloric acid or sodium hydroxide to adjust pH.

Golodirsen is an antisense oligonucleotide of the phosphorodiamidate morpholino oligomer (PMO) subclass. PMOs are synthetic molecules in which the five-membered ribofuranosyl rings found in natural DNA and RNA are replaced by a six-membered morpholino ring. Each morpholino ring is linked through an uncharged phosphorodiamidate moiety rather than the negatively charged phosphate linkage that is present in natural DNA and RNA. Each phosphorodiamidate morpholino subunit contains one of the heterocyclic bases found in DNA (adenine, cytosine, guanine, or thymine). Golodirsen contains 25 linked subunits. The sequence of bases from the 5' end to 3' end is GTTGCCTCCGTTCTGAAGGTGTTC. The molecular formula of golodirsen is  $C_{305}H_{481}N_{138}O_{112}P_{25}$  and the molecular weight is 8647.28 daltons.

The structure of golodirsen is:





## 12 CLINICAL PHARMACOLOGY

### 12.1 Mechanism of Action

Golodirsen is designed to bind to exon 53 of dystrophin pre-mRNA resulting in exclusion of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 53 skipping. Exon 53 skipping is intended to allow for production of an internally truncated dystrophin protein in patients with genetic mutations that are amenable to exon 53 skipping [see *Clinical Studies* (14)].

### 12.2 Pharmacodynamics

After treatment with VYONDYS 53, all patients evaluated (n=25) in Study 1 Part 2 [see *Clinical Studies* (14)] had an increase in skipping of exon 53 demonstrated by reverse transcription polymerase chain reaction (RT-PCR), compared to baseline.

In Study 1 Part 2 [see *Clinical Studies* (14)], dystrophin levels as assessed by the Sarepta western blot assay increased from 0.10% (SD 0.07) of normal at baseline to 1.02% (SD 1.03) of normal after 48 weeks of treatment with VYONDYS 53. The mean change from baseline in

dystrophin after 48 weeks of treatment with VYONDYS 53 was 0.92% (SD 1.01) of normal levels ( $p < 0.001$ ); the median change from baseline was 0.88%. This increase in dystrophin protein expression positively correlated with the level of exon skipping. Dystrophin levels assessed by western blot can be meaningfully influenced by differences in sample processing, analytical technique, reference materials, and quantitation methodologies. Therefore, comparing dystrophin results from different assay protocols will require a standardized reference material and additional bridging studies.

Correct localization of truncated dystrophin to the sarcolemma in muscle fibers of patients treated with golodirsen was demonstrated by immunofluorescence staining.

### **12.3 Pharmacokinetics**

The pharmacokinetics of golodirsen was evaluated in DMD patients following administration of intravenous doses ranging from 4 mg/kg/week to 30 mg/kg/week (i.e., recommended dosage). Golodirsen exposure increased proportionally with dose, with minimal accumulation with once-weekly dosing. Inter-subject variability (as %CV) for  $C_{max}$  and AUC ranged from 38% to 72%, and 34% to 44%, respectively.

#### Distribution

Steady-state volume of distribution was similar between DMD patients and healthy subjects. The mean golodirsen steady-state volume of distribution was 668 mL/kg (%CV=32.3) at a dose of 30 mg/kg. Golodirsen plasma protein binding ranged from 33% to 39% and is not concentration dependent.

#### Elimination

Golodirsen elimination half-life (SD) was 3.4 (0.6) hours, and plasma clearance was 346 mL/hr/kg at the 30 mg/kg dose.

#### Metabolism

Golodirsen is metabolically stable. No metabolites were detected in plasma or urine.

#### Excretion

Golodirsen is mostly excreted unchanged in the urine. The elimination half-life ( $t_{1/2}$ ) was 3.4 hours.

#### Specific Populations

##### *Age:*

The pharmacokinetics of golodirsen have been evaluated in male pediatric DMD patients. There is no experience with the use of VYONDYS 53 in DMD patients 65 years of age or older.

##### *Sex:*

Sex effects have not been evaluated; VYONDYS 53 has not been studied in female patients.

##### *Race:*

The potential impact of race is not known because 92% of the patients in studies were Caucasians.

*Patients with Renal Impairment:*

The effect of renal impairment on the pharmacokinetics of golodirsén was evaluated in non-DMD subjects aged 41 to 65 years with Stage 2 chronic kidney disease (CKD) (n=8, estimated glomerular filtration rate (eGFR)  $\geq 60$  and  $< 90$  mL/min/1.73 m<sup>2</sup>) or Stage 3 CKD (n=8, eGFR  $\geq 30$  and  $< 60$  mL/min/1.73 m<sup>2</sup>) and matched healthy subjects (n=8, eGFR  $\geq 90$  mL/min/1.73 m<sup>2</sup>). Subjects received a single 30 mg/kg IV dose of golodirsén.

In subjects with Stage 2 or Stage 3 CKD, exposure (AUC) increased approximately 1.2-fold and 1.9-fold, respectively. There was no change in the C<sub>max</sub> in subjects with Stage 2 CKD; in subjects with Stage 3 CKD, there was a 1.2-fold increase in C<sub>max</sub> compared with subjects with normal renal function. The effect of Stage 4 or Stage 5 CKD on golodirsén pharmacokinetics and safety has not been studied.

Estimated GFR values derived from MDRD equations and the threshold definitions for various CKD stages in otherwise healthy adults would not be generalizable to pediatric patients with DMD. Therefore, no specific dosage adjustment can be recommended for patients with renal impairment [see *Use in Specific Populations* (8.6)].

*Patients with Hepatic Impairment:*

VYONDYS 53 has not been studied in patients with hepatic impairment.

Drug Interaction Studies

Golodirsén did not inhibit CYP1A2, CYP2B6, CYP2C8, CYP2C9, CYP2C19, CYP2D6, or CYP3A4/5 *in vitro*. Golodirsén was a weak inducer of CYP1A2 and did not induce CYP2B6 or CYP3A4. Golodirsén was not metabolized by human hepatic microsomes and was not a substrate or strong inhibitor of any of the key human drug transporters tested (OAT1, OAT3, OCT2, OATP1B1, MATE1, P-gp, BCRP, and MRP2, OATP1B3 and MATE2-K). Based on *in vitro* data, golodirsén has a low potential for drug-drug interactions in humans.

## 13 NONCLINICAL TOXICOLOGY

### 13.1 Carcinogenesis, Mutagenesis, Impairment of Fertility

Carcinogenesis

Carcinogenicity studies have not been conducted with golodirsén.

Mutagenesis

Golodirsén was negative in *in vitro* (bacterial reverse mutation and chromosomal aberration in CHO cells) and *in vivo* (mouse bone marrow micronucleus) assays.

Impairment of Fertility

Fertility studies in animals were not conducted with golodirsén. No effects of golodirsén on the male reproductive system were observed following weekly subcutaneous administration (0, 120,

300, or 600 mg/kg to male mice or weekly intravenous administration (0, 80, 200, or 400 mg/kg) to male monkeys. Plasma exposure (AUC) at the highest doses tested in mouse and monkey are approximately 10 and 45 times that in humans at the recommended weekly intravenous dose of 30 mg/kg.

### **13.2 Animal Toxicology and/or Pharmacology**

Kidney toxicity was observed in studies in male mice and rats; findings in urinary bladder were observed in male mice.

In male mice, golodirsén was administered weekly for 12 weeks by intravenous injection (0, 12, 120, or 960 mg/kg) or for 26 weeks by subcutaneous injection (0, 120, 300, or 600 mg/kg). In the 12-week study, microscopic findings in kidney (tubular dilatation, basophilic or eosinophilic casts, vacuolation), correlated with increases in serum markers of renal function (e.g., urea nitrogen, creatinine), were observed primarily at the highest dose tested; hypertrophy of the transitional epithelium of the ureter or urinary bladder was observed at all doses. In the 26-week study, renal tubular degeneration and degeneration of the transitional epithelium of the urinary bladder were observed at all doses.

In male rats, intravenous administration of golodirsén (0, 60, 100, 300, or 600 mg/kg) weekly for 13 weeks resulted in tubular degeneration at all but the lowest dose tested; at the high dose, the microscopic changes were accompanied by increases in serum urea nitrogen.

In male monkeys, intravenous administration of golodirsén (0, 80, 200, or 400 mg/kg) weekly for 39 weeks resulted in microscopic changes in kidney (basophilia, dilatation, or mononuclear cell infiltration) at all doses, which correlated with increases in serum markers of renal function (urea nitrogen, creatinine) at the highest dose tested.

## **14 CLINICAL STUDIES**

The effect of VYONDYS 53 on dystrophin production was evaluated in one study in DMD patients with a confirmed mutation of the *DMD* gene that is amenable to exon 53 skipping (Study 1; NCT02310906).

Study 1 Part 1 was a double-blind, placebo-controlled, dose-titration study in 12 DMD patients. Patients were randomized 2:1 to receive VYONDYS 53 or matching placebo. VYONDYS 53-treated patients received four escalating dose levels, ranging from 4 mg/kg/week (less than the recommended dosage) to 30 mg/kg/week, by intravenous infusion for 2 weeks at each dose level.

Study 1 Part 2 was a 168-week, open-label study assessing the efficacy and safety of VYONDYS 53 at a dose of 30 mg/kg/week in the 12 patients enrolled in Part 1, plus 13 additional treatment-naïve patients with DMD amenable to exon 53 skipping. At study entry (either in Part 1 or Part 2), patients had a median age of 8 years and were on a stable dose of corticosteroids for at least 6 months. Efficacy was assessed based on change from baseline in the dystrophin protein level (measured as % of the dystrophin level in healthy subjects, i.e., % of normal) at Week 48 of Part 2. Muscle biopsies were obtained at baseline prior to treatment and at Week 48 of Part 2 in all VYONDYS 53-treated patients (n=25), and were analyzed for dystrophin protein level by Sarepta western blot. Mean dystrophin levels increased from 0.10% (SD 0.07) of normal at

baseline to 1.02% (SD 1.03) of normal by Week 48 of Study 1 Part 2, with a mean change in dystrophin of 0.92% (SD 1.01) of normal levels ( $p < 0.001$ ); the median change from baseline was 0.88%.

Individual patient dystrophin levels from Study 1 are shown in [Table 2](#).

**Table 2: Dystrophin Expression Sarepta Western Blot by Individual Patient From Study 1**

Patient Number	Sarepta Western Blot % Normal Dystrophin			Patient number	Sarepta Western Blot % Normal Dystrophin		
	Baseline	Part 2 Week 48	Change from baseline		Baseline	Part 2 Week 48	Change from baseline
1	0.08	0.09	0.01	14	0.22	0.28	0.06
2	0.11	0.11	0.01	15	0.14	0.21	0.07
3	0.21	0.22	0.01	16	0.05	0.42	0.37
4	0.05	0.12	0.08	17	0.07	1.03	0.97
5	0.03	0.12	0.09	18	0.02	1.57	1.55
6	0.06	0.14	0.09	19	0.12	1.17	1.05
7	0.12	0.37	0.25	20	0.03	1.72	1.69
8	0.11	1.06	0.95	21	0.11	1.77	1.66
9	0.06	0.54	0.48	22	0.31	4.30	3.99
10	0.05	0.97	0.92	23	0.11	0.36	0.25
11	0.06	1.55	1.49	24	0.03	0.91	0.88
12	0.07	1.91	1.84	25	0.07	1.29	1.22
13	0.10	3.25	3.15				

## 16 HOW SUPPLIED/STORAGE AND HANDLING

### 16.1 How Supplied

VYONDYS 53 injection is supplied in single dose vials. The solution is a clear to slightly opalescent, colorless liquid, and may contain trace amounts of small, white to off-white amorphous particles.

- Single-dose vials containing 100 mg/2mL (50 mg/mL) NDC 60923-465-02

### 16.2 Storage and Handling

Store VYONDYS 53 at 2°C to 8°C (36°F to 46°F). Do not freeze. Store in original carton until ready for use to protect from light.

## 17 PATIENT COUNSELING INFORMATION

### Hypersensitivity Reactions

Advise patients and/or caregivers that hypersensitivity reactions, including rash, pyrexia, pruritus, urticaria, dermatitis, and skin exfoliation have occurred in patients who were treated with VYONDYS 53. Instruct them to seek immediate medical care should they experience signs and symptoms of hypersensitivity [*see Warnings and Precautions (5.1)*].

### Kidney Toxicity

Inform patients nephrotoxicity has occurred with drugs similar to VYONDYS 53. Advise patients of the importance of monitoring for kidney toxicity by their healthcare providers during treatment with VYONDYS 53 [*see Warnings and Precautions (5.2)*].

Manufactured for:  
Sarepta Therapeutics, Inc.  
Cambridge, MA 02142 USA

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**HIGHLIGHTS OF PRESCRIBING INFORMATION**

These highlights do not include all the information needed to use ELEVIDYS safely and effectively. See full prescribing information for ELEVIDYS.

**ELEVIDYS (delandistrogene moxeparvovec-rokl) suspension, for intravenous infusion**  
Initial U.S. Approval: 2023

**INDICATIONS AND USAGE**

ELEVIDYS is an adeno-associated virus vector-based gene therapy indicated for the treatment of ambulatory pediatric patients aged 4 through 5 years with Duchenne muscular dystrophy (DMD) with a confirmed mutation in the *DMD* gene. This indication is approved under accelerated approval based on expression of ELEVIDYS micro-dystrophin in skeletal muscle observed in patients treated with ELEVIDYS. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s). (1, 12, 14)

**DOSAGE AND ADMINISTRATION**

**ELEVIDYS is for single-dose intravenous infusion only.**

- Select patients for treatment with ELEVIDYS with anti-AAVrh74 total binding antibody titers <1:400. (2.1)
- Recommended dosage:  $1.33 \times 10^{14}$  vector genomes (vg) per kg of body weight. (2.2)
- Postpone in patients with concurrent infections until the infection has resolved. (2.2)
- Assess liver function, platelet counts and troponin-I before ELEVIDYS infusion. (2)
- One day prior to infusion, initiate a corticosteroid regimen for a minimum of 60 days. Recommend modifying corticosteroid dose for patients with liver function abnormalities. (2.2)
- Administer as an intravenous infusion over 1-2 hours. Infuse at a rate of less than 10 mL/kg/hour. (2.4)

**DOSAGE FORMS AND STRENGTHS**

- ELEVIDYS is a suspension for intravenous infusion with a nominal concentration of  $1.33 \times 10^{13}$  vg/mL. (3)

- ELEVIDYS is provided in a customized kit containing ten to seventy 10 mL single-dose vials, with each kit constituting a dosage unit based on the patient's body weight. (3)

**CONTRAINDICATIONS**

- ELEVIDYS is contraindicated in patients with any deletion in exon 8 and/or exon 9 in the *DMD* gene. (4)

**WARNINGS AND PRECAUTIONS**

- **Acute Serious Liver Injury:** Acute serious liver injury has been observed. Monitor liver function before ELEVIDYS infusion, and weekly for the first 3 months after ELEVIDYS infusion. Continue monitoring until results are unremarkable. If acute serious liver injury is suspected, a consultation with a specialist is recommended. (5.1)
- **Immune-mediated Myositis:** Patients with deletions in the *DMD* gene in exons 1 to 17 and/or exons 59 to 71 may be at risk for severe immune-mediated myositis reaction. Consider additional immunomodulatory treatment (immunosuppressants [e.g., calcineurin-inhibitor] in addition to corticosteroids) if symptoms of myositis occur (e.g., unexplained increased muscle pain, tenderness, or weakness). (5.2)
- **Myocarditis:** Myocarditis and troponin-I elevations have been observed. Monitor troponin-I before ELEVIDYS infusion, and weekly for the first month after ELEVIDYS infusion. (5.3)
- **Pre-existing Immunity against AAVrh74:** Perform baseline testing for presence of anti-AAVrh74 total binding antibodies prior to ELEVIDYS administration. (5.4)

**ADVERSE REACTIONS**

Most common adverse reactions across studies (incidence  $\geq 5\%$ ) were vomiting and nausea, liver function test increased, pyrexia, and thrombocytopenia. (6.1)

**To report SUSPECTED ADVERSE REACTIONS, contact Sarepta Therapeutics, Inc., at 1-888-SAREPTA (1-888-727-3782) or FDA at 1-800-FDA-1088 or [www.fda.gov/medwatch](http://www.fda.gov/medwatch).**

**See 17 for PATIENT COUNSELING INFORMATION**

Revised: 6/2023

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\* Sections or subsections omitted from the full prescribing information are not listed.



## FULL PRESCRIBING INFORMATION

### 1 INDICATIONS AND USAGE

ELEVIDYS is indicated for the treatment of ambulatory pediatric patients aged 4 through 5 years with Duchenne muscular dystrophy (DMD) with a confirmed mutation in the *DMD* gene.

This indication is approved under accelerated approval based on expression of ELEVIDYS micro-dystrophin observed in patients treated with ELEVIDYS [see *Clinical Pharmacology* (12), *Clinical Studies* (14)]. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s).

### 2 DOSAGE AND ADMINISTRATION

**For single-dose intravenous infusion only.**

#### 2.1 Patient Selection

Select patients for treatment with ELEVIDYS with anti-AAVrh74 total binding antibody titers <1:400. An FDA-authorized test for the detection of AAVrh74 total binding antibodies is not currently available. Currently available tests may vary in accuracy and design.

#### 2.2 Dose

The recommended dose of ELEVIDYS is  $1.33 \times 10^{14}$  vector genomes per kilogram (vg/kg) of body weight (or 10 mL/kg body weight). For the number of vials required, refer to Table 7 [see *How Supplied/Storage and Handling* (16.1)].

Calculate the dose as follows:

$$\text{ELEVIDYS dose (in mL)} = \text{patient body weight (in kilogram)} \times 10$$

*The multiplication factor 10 represents the per kilogram dose ( $1.33 \times 10^{14}$  vg/kg) divided by the amount of vector genome copies per mL of the ELEVIDYS suspension ( $1.33 \times 10^{13}$  vg/mL).*

Number of ELEVIDYS vials needed = ELEVIDYS dose (in mL) divided by 10 (round to the nearest number of vials).

Example: Calculation of volume needed for a 19.6 kg patient

$$19.6 \text{ kg} \times 10 = 196 \text{ mL}$$

Number of ELEVIDYS vials needed = 196 divided by 10, rounded to the nearest number of vials = 20 vials

#### Prior to ELEVIDYS infusion:

- Due to the increased risk of serious systemic immune response, postpone ELEVIDYS in patients with infections until the infection has resolved. Clinical signs or symptoms of infection should not be evident at the time of ELEVIDYS administration [see *Patient Counseling Information* (17)].
- Assess liver function [see *Dosage and Administration* (2.4), *Warnings and Precautions* (5.1), *Use in Specific Populations* (8.6)].
- Obtain platelet count and troponin-I levels [see *Dosage and Administration* (2.4), *Warnings and Precautions* (5.3)].



- Measure baseline anti-AAVrh74 antibody titers using a Total Binding Antibody enzyme-linked immunosorbent assay (ELISA) [see *Dosage and Administration* (2), *Clinical Pharmacology* (12.6)].

ELEVIDYS administration is not recommended in patients with elevated anti-AAVrh74 total binding antibody titers ( $\geq 1:400$ ). Re-administration of ELEVIDYS is not recommended [see *Warnings and Precautions* (5.4), *Clinical Pharmacology* (12.6)].

Immune responses to the AAVrh74 vector can occur after administration of ELEVIDYS [see *Clinical Pharmacology* (12.6)]. To reduce the risk associated with an immune response, corticosteroids should be administered starting 1 day prior to ELEVIDYS infusion. Initiate a corticosteroid regimen following the appropriate schedule (see Table 1). This regimen is recommended for a minimum of 60 days after the infusion, unless earlier tapering is clinically indicated. Table 2 includes the recommended corticosteroid regimen dose modification for patients with liver function abnormalities following ELEVIDYS infusion. If acute serious liver injury is suspected, a consultation with a specialist is recommended.

For patients previously taking corticosteroids at baseline, taper off the additional peri-ELEVIDYS corticosteroids (back to baseline corticosteroid dose) over 2 weeks, or longer as needed. For patients not previously taking corticosteroids at baseline, taper the added peri-ELEVIDYS corticosteroids off (back to no corticosteroids) over 4 weeks, or longer, as needed, and the corticosteroids should not be stopped abruptly.

**Table 1: Recommended pre- and post-infusion corticosteroid dosing**

Baseline corticosteroid dosing <sup>a</sup>	Peri-ELEVIDYS infusion corticosteroid dose (prednisone equivalent) <sup>b</sup>	Recommended maximum total daily dose (prednisone equivalent) <sup>b</sup>
Daily or intermittent dose	Start <b>1 day</b> prior to infusion: 1 mg/kg/day (and continue baseline dose)	60 mg/day
High dose for 2 days per week	Start <b>1 day</b> prior to infusion: 1 mg/kg/day taken on days without high-dose corticosteroid treatment (and continue baseline dose)	60 mg/day
Not on corticosteroids	Start <b>1 week</b> prior to infusion: 1.5 mg/kg/day	60 mg/day

<sup>a</sup> Patient continues to receive this dose

<sup>b</sup> Deflazacort is not recommended for use as a peri-ELEVIDYS infusion corticosteroid

**Table 2: Recommended corticosteroid regimen dose modification for liver function abnormalities following ELEVIDYS infusion<sup>a</sup>**

Peri-ELEVIDYS infusion corticosteroid dosing	Modified corticosteroid dose following ELEVIDYS infusion (prednisone equivalent) <sup>b</sup>	Recommended maximum total daily dose (prednisone equivalent) <sup>b</sup>
Baseline + 1 mg/kg/day	Increase to 2 mg/kg/day (and continue baseline dose)	120 mg/day
Baseline + 1 mg/kg/day taken on days without high-dose corticosteroid treatment	Increase to 2 mg/kg/day taken on days without high-dose corticosteroid treatment (and continue baseline dose)	120 mg/day
1.5 mg/kg/day	Increase from 1.5 mg/kg/day to 2.5 mg/kg/day	120 mg/day

<sup>a</sup> GGT  $\geq$  150 U/L and/or other clinically significant liver function abnormalities (e.g., total bilirubin  $> 2 \times$  ULN) following infusion. For GGT or bilirubin elevations that do not respond to these oral corticosteroid increases, IV bolus corticosteroids may be considered.

<sup>b</sup> Deflazacort is not recommended for use as a peri-ELEVIDYS infusion corticosteroid

## 2.3 Preparation

### General precautions

- Prepare ELEVIDYS using aseptic technique.
- Verify the required dose of ELEVIDYS based on the patient's body weight.
- Confirm that the kit contains sufficient number of vials to prepare the ELEVIDYS infusion for the patient.
- Visually inspect parenteral drug products for particulate matter and discoloration prior to administration, whenever suspension and container permit. ELEVIDYS may contain white to off-white particles.

### Recommended supplies and materials:

- 60 mL siliconized polypropylene syringes
- 21-gauge or smaller stainless steel needles

### Preparing ELEVIDYS infusion

1. Thaw ELEVIDYS before use.
  - When thawed in the refrigerator, ELEVIDYS vials are stable for up to 14 days in the refrigerator (2°C to 8°C [36° F to 46° F]) when stored in the upright position.
  - Frozen ELEVIDYS vials will thaw in approximately 2 hours when placed at room temperature (up to 25°C [77°F]) when removed from original packaging.
2. Inspect vials to ensure no ice crystals are present prior to preparation.
3. When thawed, swirl gently.
  - Do not shake.
  - Do not refreeze.
  - Do not place back in the refrigerator.
4. Visually inspect each vial of ELEVIDYS. ELEVIDYS is a clear, colorless liquid that may have some opalescence. ELEVIDYS may contain white to off-white particles.
  - Do not use if the suspension in the vials is cloudy or discolored.
5. Remove the plastic flip-off cap from the vials and disinfect the rubber stopper with a sterilizing agent (e.g., alcohol wipes).
6. Withdraw 10 mL of ELEVIDYS from each vial provided in the customized ELEVIDYS kit (refer to Table 7).
  - Do not use filter needles during preparation of ELEVIDYS.
  - Multiple syringes will be required to withdraw the required volume.
  - Remove air from the syringes and cap the syringes.
7. Maintain syringes at room temperature prior to and during administration.
  - Sealed ELEVIDYS thawed vials are stable up to 24 hours at room temperature (up to 25°C [77°F]) when stored in upright position.

## 2.4 Administration

### Recommended supplies and materials:

- Syringe infusion pump
- 0.2-micron PES\* in-line filter
- PVC\* (non-DEHP\*), polyurethane IV infusion tubing and catheter

\*PVC = Polyvinyl chloride, DEHP = Di(2-ethylhexyl) phthalate, PES = Polyether sulfone

### Administer ELEVIDYS as a single-dose intravenous infusion through a peripheral venous catheter:

Consider application of a topical anesthetic to the infusion site prior to administration of IV insertion.

Recommend inserting a back-up catheter.

1. Flush the intravenous access line prior to the ELEVIDYS infusion at the same infusion rate.
2. Administer ELEVIDYS via intravenous infusion using a syringe infusion pump with an in-line 0.2-micron filter at a duration of approximately 1 to 2 hours, or longer at care team discretion, through a peripheral limb vein.
3. Infuse at a rate of less than 10 mL/kg/hour.
  - Do not administer ELEVIDYS as an intravenous push.
  - Do not infuse ELEVIDYS in the same intravenous access line with any other product.
  - Use ELEVIDYS within 4 hours after drawing into syringe. Discard the ELEVIDYS-containing syringe(s) if infusion of the drug has not started within the 4-hour timeframe.
4. Flush the intravenous access line with 0.9% Sodium Chloride Injection after the ELEVIDYS infusion.
  - Discard unused ELEVIDYS [see *How Supplied/Storage and Handling* (16.2)].
  - Dispose of the needle and syringe [see *How Supplied/Storage and Handling* (16.2)].

### Monitoring Post-ELEVIDYS Administration

- Assess liver function (clinical exam, GGT, and total bilirubin) weekly for the first 3 months. Continue monitoring if clinically indicated, until results are unremarkable (normal clinical exam, GGT and total bilirubin levels return to near baseline levels) [see *Warning and Precautions* (5.1), *Specific Populations* (8.6)].
- Obtain platelet counts weekly for the first two weeks [see *Adverse Reactions* (6.1)]. Continue monitoring if clinically indicated.
- Measure troponin-I weekly for the first month [see *Warning and Precautions* (5.3)]. Continue monitoring if clinically indicated.

## 3 DOSAGE FORMS AND STRENGTHS

ELEVIDYS is a preservative-free, sterile, clear, colorless liquid that may have some opalescence and may contain white to off-white particles.

ELEVIDYS is a suspension for intravenous infusion with a nominal concentration of  $1.33 \times 10^{13}$  vg/mL.

ELEVIDYS is provided in a customized kit containing ten to seventy 10 mL single-dose vials, with each kit constituting a dosage unit based on the patient's body weight [see *How Supplied/Storage and Handling* (16.1)].

The intravenous dosage is determined by patient body weight, with a recommended dose of  $1.33 \times 10^{14}$  vector genomes (vg)/kg.

## 4 CONTRAINDICATIONS

ELEVIDYS is contraindicated in patients with any deletion in exon 8 and/or exon 9 in the *DMD* gene [see *Warnings and Precautions* (5.2)].

## 5 WARNINGS AND PRECAUTIONS

### 5.1 Acute Serious Liver Injury

Acute serious liver injury has been observed with ELEVIDYS. Administration of ELEVIDYS may result in elevations of liver enzymes (e.g., GGT, ALT) and total bilirubin, typically seen within 8 weeks.

Patients with preexisting liver impairment, chronic hepatic condition or acute liver disease (e.g., acute hepatic viral infection) may be at higher risk of acute serious liver injury. Postpone ELEVIDYS administration in patients with acute liver disease until resolved or controlled. Patients with hepatic impairment, acute liver disease, chronic hepatic condition or elevated GGT have not been studied in clinical trials with ELEVIDYS [see *Specific Populations* (8.6)].

In clinical studies, liver function test increased (including increases in GGT, GLDH, ALT, AST, or total bilirubin) was commonly reported typically within 8 weeks following ELEVIDYS infusion, with the majority of cases being asymptomatic [see *Adverse Reactions* (6.1)]. Cases resolved spontaneously or with systemic corticosteroids and resolved without clinical sequelae within 2 months. No cases of liver failure were reported.

Prior to ELEVIDYS administration, perform liver enzyme test [see *Dosage and Administration* (2.2)]. Monitor liver function (clinical exam, GGT, and total bilirubin) weekly for the first 3 months following ELEVIDYS infusion. Continue monitoring if clinically indicated, until results are unremarkable (normal clinical exam, GGT and total bilirubin levels return to near baseline levels) [see *Dosage and Administration* (2.4)].

Systemic corticosteroid treatment is recommended for patients before and after ELEVIDYS infusion [see *Dosage and Administration* (2.2)]. Adjust corticosteroid regimen when indicated [see *Dosage and Administration* (2.2)]. If acute serious liver injury is suspected, a consultation with a specialist is recommended.

### 5.2 Immune-mediated Myositis

In clinical trials, immune-mediated myositis has been observed approximately 1 month following ELEVIDYS infusion in patients with deletion mutations involving exon 8 and/or exon 9 in the *DMD* gene. Symptoms of severe muscle weakness, including dysphagia, dyspnea and hypophonia, were observed. In a life-threatening case of immune-mediated myositis, symptoms resolved during hospitalization following additional immunomodulatory treatment; muscle strength gradually improved but did not return to baseline level. These immune reactions may be due to a T-cell based response from lack of self-tolerance to a specific region encoded by the transgene corresponding to exons 1-17 of the *DMD* gene.

Limited data are available for ELEVIDYS treatment in patients with mutations in the *DMD* gene in exons 1 to 17 and/or exons 59 to 71 [see *Clinical Studies* (14)]. Patients with deletions in these regions may be at risk for a severe immune-mediated myositis reaction. ELEVIDYS is contraindicated in patients with any deletion in exon 8 and/or exon 9 in the *DMD* gene due to the increased risk for a severe immune-mediated myositis reaction [see *Contraindications* (4)].

Advise patients to contact a physician immediately if they experience any unexplained increased muscle pain, tenderness, or weakness, including dysphagia, dyspnea or hypophonia as these may be symptoms of myositis. Consider additional immunomodulatory treatment (immunosuppressants [e.g., calcineurin-inhibitor] in addition to corticosteroids) based on patient's clinical presentation and medical history if these symptoms occur.

### 5.3 Myocarditis

Acute serious myocarditis and troponin-I elevations have been observed following ELEVIDYS infusion in clinical trials.

Monitor troponin-I before ELEVIDYS infusion and weekly for the first month following infusion [see *Dosage and Administration* (2.4)]. Continue monitoring if clinically indicated. More frequent monitoring may be warranted in the presence of cardiac symptoms, such as chest pain or shortness of breath.

Advise patients to contact a physician immediately if they experience cardiac symptoms.

### 5.4 Pre-existing Immunity against AAVrh74

In AAV-vector based gene therapies, preexisting anti-AAV antibodies may impede transgene expression at desired therapeutic levels. Following treatment with ELEVIDYS all subjects developed anti-AAVrh74 antibodies. Perform baseline testing for the presence of anti-AAVrh74 total binding antibodies prior to ELEVIDYS administration [see *Dosage and Administration* (2.1)].

ELEVIDYS administration is not recommended in patients with elevated anti-AAVrh74 total binding antibody titers ( $\geq 1:400$ ).

## 6 ADVERSE REACTIONS

The most common adverse reactions (incidence  $\geq 5\%$ ) reported in clinical studies were vomiting, nausea, liver function test increased, pyrexia, and thrombocytopenia.

The following clinically significant adverse reactions are described elsewhere in the labeling:

- Acute serious liver injury [see *Warnings and Precautions* (5.1)]
- Immune-mediated myositis [see *Warnings and Precautions* (5.2)]
- Myocarditis [see *Warnings and Precautions* (5.3)]

### 6.1 Clinical Trials Experience

Because clinical trials are conducted under widely varying conditions, adverse reaction rates observed in the clinical trials of a drug cannot be directly compared to rates in the clinical trials of another drug and may not reflect the rates observed in practice.

The safety data described in this section reflect exposure to a one-time intravenous infusion of ELEVIDYS in 85 male subjects with a confirmed mutation of the *DMD* gene in three on-going clinical studies, including two open-label studies and one study that included a double-blind, placebo-controlled period. Prior to ELEVIDYS infusion, patients in the ELEVIDYS treatment group had a mean age of 7.08 years (range: 3 to 20) and mean weight of 25.91 kg (range: 12.5 to 80.1). 73 subjects received the recommended dose of  $1.33 \times 10^{14}$  vg/kg, and 12 received a lower dose. Table 3 below presents adverse reactions from these three clinical studies.

The most common adverse reactions (incidence  $\geq 5\%$ ) across all studies are summarized in Table 3.

Adverse reactions were typically seen within the first 2 weeks (nausea, vomiting, thrombocytopenia, pyrexia), or within the first 2 months (immune-mediated myositis, liver function test increased). Vomiting may occur as early as on the day of the infusion.

**Table 3. Adverse reactions (Incidence  $\geq 5\%$ ) following treatment with ELEVIDYS in Clinical Studies**

Adverse reactions	ELEVIDYS (N=85) %
Vomiting	61
Nausea	40
Liver function test increased <sup>a</sup>	37
Pyrexia	24
Thrombocytopenia <sup>b</sup>	12

<sup>a</sup> Includes: AST increased, ALT increased, GGT increased, GLDH increased, hepatic enzyme increased, transaminases increased, blood bilirubin increased

<sup>b</sup> Transient, mild, asymptomatic decrease in platelet counts

In the double-blind, placebo-controlled trial (Study 1 Part 1), subjects 4 to 7 years of age (N=41) received either ELEVIDYS (N=20) at the recommended dose of  $1.33 \times 10^{14}$  vg/kg (n=8) or lower dose (n=12) or received placebo (N=21). Table 4 below presents the most frequent adverse reactions from Study 1 Part 1.

**Table 4. Adverse reactions occurring in ELEVIDYS-treated subjects and at least 10% more frequently than in placebo in Study 1, Part 1**

Adverse reactions	ELEVIDYS (N=20) %	Placebo (N=21) %
Vomiting	65	33
Nausea	35	10
Liver function test increased <sup>a</sup>	25	0
Pyrexia	20	5

<sup>a</sup> Includes: AST increased, ALT increased, GGT increased, GLDH increased, hepatic enzyme increased, transaminases increased, blood bilirubin increased.

## 7 DRUG INTERACTIONS

Prior to initiating the corticosteroid regimen required before ELEVIDYS administration, consider the patient's vaccination status. Patients should, if possible, be brought up-to-date with all immunizations in agreement with current immunization guidelines. Vaccinations should be completed at least 4 weeks prior to initiation of the corticosteroid regimen.

## 8 USE IN SPECIFIC POPULATIONS

### 8.1 Pregnancy

#### Risk Summary

ELEVIDYS is not intended for use in pregnant women.

In the U.S. general population, the estimated background risks of major birth defects and miscarriage in clinically recognized pregnancies is 2% to 4% and 15% to 20%, respectively.

### 8.2 Lactation

#### Risk Summary

There is no information available on the presence of ELEVIDYS in human milk, the effects on the breastfed infant, or the effects on milk production.

### 8.4 Pediatric Use

ELEVIDYS is indicated for the treatment of ambulatory pediatric patients 4 through 5 years of age with Duchenne muscular dystrophy with a confirmed mutation in the *DMD* gene. This indication is based on



expression of ELEVIDYS micro-dystrophin protein in skeletal muscle observed in DMD patients treated with ELEVIDYS. The effectiveness and safety of ELEVIDYS has not been established in pediatric patients younger than 3 years of age. The effectiveness of ELEVIDYS has not been established in pediatric patients 3 years of age and in pediatric patients 6 years of age and older [see *Clinical Pharmacology* (12.2), *Clinical Studies* (14)].

## 8.5 Geriatric Use

The safety and efficacy of ELEVIDYS in geriatric patients with DMD have not been studied.

## 8.6 Hepatic Impairment

The safety and efficacy of ELEVIDYS in patients with hepatic impairment or elevated GGT have not been studied.

Postpone ELEVIDYS administration in patients with acute liver disease until resolved or controlled. ELEVIDYS therapy should be carefully considered in patients with preexisting liver impairment or chronic hepatic viral infection. These patients may be at increased risk of acute serious liver injury [see *Warnings and Precautions* (5.1)].

In clinical trials, liver function test increase was commonly reported in subjects following ELEVIDYS infusion [see *Warnings and Precautions* (5.1), *Adverse Reactions* (6.1)].

## 11 DESCRIPTION

ELEVIDYS (delandistrogene moxeparvovec-rokl) is a recombinant gene therapy designed to deliver the gene encoding the ELEVIDYS micro-dystrophin protein. ELEVIDYS is a non-replicating, recombinant, adeno-associated virus serotype rh74 (AAVrh74) based vector containing the ELEVIDYS micro-dystrophin transgene under the control of the MHCK7 promoter. The genome within the ELEVIDYS AAVrh74 vector contains no viral genes and consequently is incapable of replication or reversion to a replicating form. The micro-dystrophin protein expressed by ELEVIDYS is a shortened version (138 kDa, compared to 427 kDa size of dystrophin expressed in normal muscle cells) that contains selected domains of dystrophin expressed in normal muscle cells.

ELEVIDYS is a preservative-free, sterile, clear, colorless liquid that may have some opalescence and may contain white to off-white particles. ELEVIDYS is a suspension for intravenous infusion with a nominal concentration of  $1.33 \times 10^{13}$  vg/mL and supplied in a single-dose 10 mL vial. Each vial contains an extractable volume of 10 mL and the following excipients: 200mM sodium chloride, 13 mM tromethamine HCl, 7 mM tromethamine, 1mM magnesium chloride, 0.001% poloxamer 188.

## 12 CLINICAL PHARMACOLOGY

### 12.1 Mechanism of Action

ELEVIDYS is the recombinant gene therapy product that is comprised of a non-replicating, recombinant, adeno-associated virus (AAV) serotype rh74 (AAVrh74) capsid and a ssDNA expression cassette flanked by inverted terminal repeats (ITRs) derived from AAV2. The cassette contains: 1) an MHCK7 gene regulatory component comprising a creatine kinase 7 promoter and an  $\alpha$ -myosin heavy chain enhancer, and 2) the DNA transgene encoding the engineered ELEVIDYS micro-dystrophin protein.

**Vector/Capsid:** Clinical and nonclinical studies have demonstrated AAVrh74 serotype transduction in skeletal muscle cells. Additionally, in nonclinical studies, AAVrh74 serotype transduction has been demonstrated in cardiac and diaphragm muscle cells.

**Promoter:** The MHCK7 promoter/enhancer drives transgene expression and has been shown in animal models to drive transgenic ELEVIDYS micro-dystrophin protein expression predominantly in skeletal muscle (including

diaphragm) and cardiac muscle. In clinical studies, muscle biopsy analyses have confirmed ELEVIDYS micro-dystrophin expression in skeletal muscle.

Transgene: DMD is caused by a mutation in the *DMD* gene resulting in lack of functional dystrophin protein. ELEVIDYS carries a transgene encoding a micro-dystrophin protein consisting of selected domains of dystrophin expressed in normal muscle cells.

ELEVIDYS micro-dystrophin has been demonstrated to localize to the sarcolemma.

## 12.2 Pharmacodynamics

In 61 subjects who received ELEVIDYS in clinical studies, ELEVIDYS micro-dystrophin protein expression from muscle biopsies (gastrocnemius) was quantified by western blot and localized by immunofluorescence staining (fiber intensity and percentage ELEVIDYS micro-dystrophin).

ELEVIDYS micro-dystrophin expression (expressed as change from baseline) as measured by western blot was the primary objective of Study 1 and Study 2. Muscle biopsies were obtained at baseline prior to ELEVIDYS infusion and at Week 12 after ELEVIDYS infusion in all subjects. The absolute quantity of ELEVIDYS micro-dystrophin was measured by western blot assay, adjusted by muscle content and expressed as a percent of control (levels of wild-type dystrophin in subjects without DMD or Becker muscular dystrophy) in muscle biopsy samples. Results of subjects receiving  $1.33 \times 10^{14}$  vg/kg ELEVIDYS are presented in Table 5.

**Table 5: ELEVIDYS Micro-Dystrophin Expression in Studies 1 and 2 (Western Blot Assay)<sup>abcd</sup>**

Western blot (% of ELEVIDYS micro-dystrophin compared to control)	Study 1 (Week 12) Part 1 (n = 6)	Study 1 (Week 12) Part 2 (n=21)	Study 2 (Week 12) Cohort 1 (n = 20)
Mean change from baseline (SD)	43.4 (48.6)	40.7 (32.3)	54.2 (42.6)
Median change from baseline (Min, Max)	24.3 (1.6, 116.3)	40.8 (0.0, 92.0)	50.6 (4.8, 153.9)

<sup>a</sup> All patients received  $1.33 \times 10^{14}$  vg/kg, as measured by ddPCR

<sup>b</sup> Muscle biopsies were obtained from the gastrocnemius

<sup>c</sup> Change from baseline was statistically significant

<sup>d</sup> Adjusted for muscle content. Control was level of wild-type (normal) dystrophin in normal muscle.

For subjects aged 4 through 5 years who received  $1.33 \times 10^{14}$  vg/kg of ELEVIDYS, the mean (SD) ELEVIDYS micro-dystrophin expression levels (change from baseline) at Week 12 following ELEVIDYS infusion were 95.7% (N=3, SD: 17.9%) in Study 1 Parts 1 and 2 and 51.7% (N=11, SD: 41.0%) in Study 2 Cohort 1.

Assessment of ELEVIDYS micro-dystrophin levels can be meaningfully influenced by differences in sample processing, analytical technique, reference materials, and quantitation methodologies. Therefore, valid comparisons of ELEVIDYS micro-dystrophin measurements obtained from different assays cannot be made.

## 12.3 Pharmacokinetics

### Vector Distribution and Vector Shedding

#### Nonclinical Data

Biodistribution of ELEVIDYS was evaluated in tissue samples collected from healthy mice and *Dmd*<sup>mdx</sup> mice following intravenous administration in toxicology studies. At 12 weeks following ELEVIDYS administration at



dose levels of  $1.33 \times 10^{14}$  to  $4.02 \times 10^{14}$  vg/kg, vector DNA was detected in all major organs with the highest quantities detected in the liver, followed by lower levels in the heart, adrenal glands, skeletal muscle, and aorta. ELEVIDYS was also detected at low levels in the spinal cord, sciatic nerve and gonads (testis). Protein expression of ELEVIDYS micro-dystrophin was highest in cardiac tissue, exceeding physiologic dystrophin expression levels in healthy mice, with lower levels in the skeletal muscle and diaphragm. In some studies, micro-dystrophin was also detected at low levels in the liver.

### Clinical Data

Following IV administration, ELEVIDYS vector genome undergoes distribution via systemic circulation and distributes into target muscle tissues followed by elimination in the urine and feces. ELEVIDYS biodistribution and tissue transduction are detected in the target muscle tissue groups and quantified in the gastrocnemius or biceps femoris biopsies obtained from patients with mutations in the *DMD* gene. Evaluation of ELEVIDYS vector genome exposure in clinical muscle biopsies at Week 12 post-dose expressed as copies per nucleus revealed ELEVIDYS drug distribution and transduction with a mean change from baseline of 2.91 and 3.44 copies per nucleus at the recommended dose of  $1.33 \times 10^{14}$  vg/kg for Study 1 and Study 2 Cohort 1, respectively.

In Study 2 Cohort 1, the biodistribution and vector shedding of ELEVIDYS in the serum and excreta were quantified, respectively. The mean maximum concentration ( $C_{max}$ ) in the serum was  $0.0049 \times 10^{13}$  copies/mL and  $4.11 \times 10^5$  copies/mL in the urine,  $4.72 \times 10^7$  copies/mL in the saliva, and  $2.32 \times 10^7$  copies/ $\mu$ g in the feces. The median time to achieve maximum concentration ( $T_{max}$ ) was 5.3 hours post-dose in the serum, followed by 6.7 hours, 6.4 hours and 13.5 days post-dose in the saliva, urine, and feces, respectively. The median time to achieve first below limit of quantification (BLOQ) sample followed by 2 consecutive BLOQ samples were 63 days post-dose for serum. The median time to achieve complete elimination as the first below limit of detection (BLOD) sample followed by 2 consecutive BLOD samples were 49.8 days, 123 days and 162 days post-dose for saliva, urine and feces, respectively. The estimated elimination half-life of ELEVIDYS vector genome in the serum is approximately 12 hours, and the majority of the drug is expected to be cleared from the serum by 1-week post-dose. In the excreta, the estimated elimination half-life of ELEVIDYS vector genome is 40 hours, 55 hours, and 60 hours in the urine, feces, and saliva, respectively. As an AAV-based gene therapy that consists of a protein capsid containing the transgene DNA genome of interest, ELEVIDYS capsid proteins are broken down through proteasomal degradation following AAV entry into target cells. As such, ELEVIDYS is not likely to exhibit the drug-drug interaction potential mediated by known drug metabolizing enzymes (cytochrome P450-based) and drug transporters.

## **12.6 Immunogenicity**

The observed incidence of anti-AAVrh74 antibodies is highly dependent on the sensitivity and specificity of the assay. Differences in assay methods preclude meaningful comparisons of the incidence of anti-AAVrh74 antibodies in the studies described below with the incidence of anti-AAVrh74 antibodies in other studies.

In ELEVIDYS clinical studies, patients were required to have baseline anti-AAVrh74 total binding antibodies of  $\leq 1:400$ , measured using an investigational total binding antibody enzyme-linked immunosorbent assay (ELISA), and only patients with baseline anti-AAVrh74 total binding antibodies  $< 1:400$  were enrolled in those studies. The safety and efficacy of ELEVIDYS in patients with elevated anti-AAVrh74 total binding antibody titer ( $\geq 1:400$ ) have not been evaluated [see *Clinical Studies* (14)].

Across clinical studies evaluating a total of 84 patients, elevated anti-AAVrh74 total binding antibodies titers were observed in all patients following a one-time ELEVIDYS infusion. Anti-AAVrh74 total binding antibody titers reached at least 1:409,600 in every subject, and the maximum titers exceeded 1:26,214,400 in certain subjects. The safety of re-administration of ELEVIDYS or any other AAVrh74 vector-based gene therapy in the presence of high anti-AAVrh74 total binding antibody titer has not been evaluated in humans [see *Warnings and Precautions* (5.4)].

## 13 NONCLINICAL TOXICOLOGY

### 13.1 Carcinogenesis, Mutagenesis, Impairment of Fertility

No animal studies have been performed to evaluate the effects of ELEVIDYS on carcinogenicity, mutagenesis, or impairment of fertility.

## 14 CLINICAL STUDIES

Accelerated approval was primarily based on data from Study 1 and Study 2 described below.

Study 1 is an ongoing multi-center study including:

- Part 1: a 48-week, randomized, double-blind, placebo-controlled period
- Part 2: a 48-week period that began following completion of Part 1. Patients who received placebo during Part 1 were treated with ELEVIDYS, and patients treated with ELEVIDYS during Part 1 received placebo.

The study population consisted of male ambulatory DMD patients (N=41) aged 4 through 7 years with either a confirmed frameshift mutation, or a premature stop codon mutation between exons 18 to 58 in the *DMD* gene.

Patients were randomized 1:1 to receive either ELEVIDYS (N=20) or placebo (N=21), as a single intravenous infusion via a peripheral limb. Randomization was stratified by age (i.e., aged 4 to 5 years vs. aged 6 to 7 years). In the ELEVIDYS group, eight patients received  $1.33 \times 10^{14}$  vg/kg of ELEVIDYS, and 12 patients received lower doses. Key demographic and baseline characteristics are presented in Table 6 below.

**Table 6: Key Demographic and Baseline Characteristics (Part 1)**

Characteristic	All (n=41)	ELEVIDYS (n=20)	Placebo (n=21)	ELEVIDYS Aged 4 through 5-year-old subgroup (n=8)	Placebo Aged 4 through 5-year-old subgroup (n=8)
Race group White (%)	73.2	65	81	75	100
Mean age [range] (years)	6.27 [4.34 – 7.98]	6.29 [4.47 – 7.85]	6.24 [4.34 – 7.98]	4.98 [4.47 – 5.39]	5.15 [4.93 – 5.91]
Mean weight [range] (kg)	22.4 [15.0 – 34.5]	23.3 [18.0 – 34.5]	21.6 [15.0 – 30.0]	20.1 [18.0 – 23.5]	19.8 [15.0 – 21.5]
Mean NSAA total score [range]	21.2 [13 – 29]	19.8 [13 – 26]	22.6 [15 – 29]	20.1 [17 – 23]	20.4 [15 – 24]
Mean time to rise from floor [range] (seconds)	4.3 [2.7 – 10.4]	5.1 [3.2 – 10.4]	3.6 [2.7 – 4.8]	3.9 [3.2 – 5.2]	3.8 [3.2 – 10.4]

All subjects were on a stable dose of corticosteroids for DMD for at least 12 weeks prior to ELEVIDYS infusion. All randomized subjects had baseline anti-AAVrh74 antibody titers <1:400 as determined by an investigational total binding antibody ELISA.

One day prior to treatment with ELEVIDYS or placebo, the subject's background dose of corticosteroid for DMD was increased to at least 1 mg/kg of a corticosteroid (prednisone equivalent) daily and was continued at this level for at least 60 days after the infusion, unless earlier tapering was clinically indicated.

The primary objectives of Study 1 were to evaluate expression of ELEVIDYS micro-dystrophin in skeletal muscle, and to evaluate the effect of ELEVIDYS on the North Star Ambulatory Assessment (NSAA) total score.

Results of ELEVIDYS micro-dystrophin measured by western blot are presented in Table 5 [see *Clinical Pharmacology* (12.2)].

The change in NSAA total score was assessed from baseline to Week 48 after infusion of ELEVIDYS or placebo. The difference between the ELEVIDYS and placebo groups was not statistically significant ( $p=0.37$ ). The least squares (LS) mean changes in NSAA total score from baseline to Week 48 was 1.7 (standard error [SE]: 0.6) points for the ELEVIDYS group and 0.9 (SE: 0.6) points for the placebo group.

Exploratory subgroup analyses showed that for subjects aged 4 through 5 years, the LS mean changes (SE) in NSAA total score from baseline to Week 48 were 4.3 (0.7) points for the ELEVIDYS group, and 1.9 (0.7) points for the placebo group, a numerical advantage for ELEVIDYS. For subjects aged 6 through 7 years, the LS mean changes (SE) in NSAA total score from baseline to Week 48 were -0.2 (0.7) points for the ELEVIDYS group and 0.5 (0.7) points for the placebo group, a numerical disadvantage for ELEVIDYS.

Study 2 is an ongoing, open-label, multi-center study which includes a cohort of 20 ambulatory male DMD subjects aged 4 through 7 years. All 20 subjects have a confirmed frameshift mutation, canonical splice site mutation, or premature stop codon mutation in the *DMD* gene.

At study entry, 75% of subjects were white with a mean age of 5.81 years (range: 4.38 to 7.94), mean weight of 21.2 kg (range: 15.2 to 33.1), mean NSAA total score of 22.1 points (range: 18 to 26), and mean time to rise from floor of 4.2 seconds (range: 2.4 to 8.2). Subjects received corticosteroids for DMD before infusion according to Table 1 [see *Dosage and Administration* (2.2)]. All subjects had baseline anti-AAVrh74 antibodies titers <1:400 as determined by the investigational total binding antibody ELISA and received a single intravenous infusion of  $1.33 \times 10^{14}$  vg/kg ELEVIDYS.

The primary objective of the study was to evaluate the effect of ELEVIDYS micro-dystrophin expression as measured by western blot. Results are presented in Table 5 [see *Clinical Pharmacology* (12.2)].

## 16 HOW SUPPLIED/STORAGE AND HANDLING

### 16.1 How Supplied

ELEVIDYS is shipped frozen ( $\leq -60^{\circ}\text{C}$  [ $-76^{\circ}\text{F}$ ]) in 10 mL vials.

ELEVIDYS is supplied as a customized kit to meet dosing requirements for each patient [see *Dosage and Administration* (2.1)]. Each kit contains:

- Ten (10) to seventy (70) single-dose vials of ELEVIDYS
- One alcohol wipe per vial

Each ELEVIDYS pack may contain a maximum of two different drug product lots.

The total number of vials in each kit corresponds to the dosing requirement for the individual patient, based on the patient's body weight, and is specified on the package [see *Dosage and Administration* (2.2)]. Each kit includes a specified number of ELEVIDYS vials (with a minimum of 10 vials for a patient with 10.0 – 10.4 kg body weight range, and a maximum of 70 vials for a patient with body weight of 69.5 kg and above). Kit sizes and National Drug Codes (NDC) are provided in Table 7.

**Table 7: ELEVIDYS Multi-vial Kits**

Patient Weight (kg)	Total Vials per Kit	Total Dose Volume per Kit (mL)	NDC Number
10.0 – 10.4	10	100	60923-501-10
10.5 – 11.4	11	110	60923-502-11

<b>Patient Weight (kg)</b>	<b>Total Vials per Kit</b>	<b>Total Dose Volume per Kit (mL)</b>	<b>NDC Number</b>
11.5 – 12.4	12	120	60923-503-12
12.5 – 13.4	13	130	60923-504-13
13.5 – 14.4	14	140	60923-505-14
14.5 – 15.4	15	150	60923-506-15
15.5 – 16.4	16	160	60923-507-16
16.5 – 17.4	17	170	60923-508-17
17.5 – 18.4	18	180	60923-509-18
18.5 – 19.4	19	190	60923-510-19
19.5 – 20.4	20	200	60923-511-20
20.5 – 21.4	21	210	60923-512-21
21.5 – 22.4	22	220	60923-513-22
22.5 – 23.4	23	230	60923-514-23
23.5 – 24.4	24	240	60923-515-24
24.5 – 25.4	25	250	60923-516-25
25.5 – 26.4	26	260	60923-517-26
26.5 – 27.4	27	270	60923-518-27
27.5 – 28.4	28	280	60923-519-28
28.5 – 29.4	29	290	60923-520-29
29.5 – 30.4	30	300	60923-521-30
30.5 – 31.4	31	310	60923-522-31
31.5 – 32.4	32	320	60923-523-32
32.5 – 33.4	33	330	60923-524-33
33.5 – 34.4	34	340	60923-525-34
34.5 – 35.4	35	350	60923-526-35
35.5 – 36.4	36	360	60923-527-36
36.5 – 37.4	37	370	60923-528-37
37.5 – 38.4	38	380	60923-529-38
38.5 – 39.4	39	390	60923-530-39
39.5 – 40.4	40	400	60923-531-40

<b>Patient Weight (kg)</b>	<b>Total Vials per Kit</b>	<b>Total Dose Volume per Kit (mL)</b>	<b>NDC Number</b>
40.5 – 41.4	41	410	60923-532-41
41.5 – 42.4	42	420	60923-533-42
42.5 – 43.4	43	430	60923-534-43
43.5 – 44.4	44	440	60923-535-44
44.5 – 45.4	45	450	60923-536-45
45.5 – 46.4	46	460	60923-537-46
46.5 – 47.4	47	470	60923-538-47
47.5 – 48.4	48	480	60923-539-48
48.5 – 49.4	49	490	60923-540-49
49.5 – 50.4	50	500	60923-541-50
50.5 – 51.4	51	510	60923-542-51
51.5 – 52.4	52	520	60923-543-52
52.5 – 53.4	53	530	60923-544-53
53.5 – 54.4	54	540	60923-545-54
54.5 – 55.4	55	550	60923-546-55
55.5 – 56.4	56	560	60923-547-56
56.5 – 57.4	57	570	60923-548-57
57.5 – 58.4	58	580	60923-549-58
58.5 – 59.4	59	590	60923-550-59
59.5 – 60.4	60	600	60923-551-60
60.5 – 61.4	61	610	60923-552-61
61.5 – 62.4	62	620	60923-553-62
62.5 – 63.4	63	630	60923-554-63
63.5 – 64.4	64	640	60923-555-64
64.5 – 65.4	65	650	60923-556-65
65.5 – 66.4	66	660	60923-557-66
66.5 – 67.4	67	670	60923-558-67
67.5 – 68.4	68	680	60923-559-68
68.5 – 69.4	69	690	60923-560-69

Patient Weight (kg)	Total Vials per Kit	Total Dose Volume per Kit (mL)	NDC Number
69.5 and above	70	700	60923-561-70

An ELEVIDYS 10 mL single-dose vial (NDC 60923-500-01) is not sold individually.

## 16.2 Storage and Handling

- ELEVIDYS is shipped and delivered at  $\leq -60^{\circ}\text{C}$  [ $-76^{\circ}\text{F}$ ].
- ELEVIDYS can be refrigerated for up to 14 days when stored at  $2^{\circ}\text{C}$  to  $8^{\circ}\text{C}$  ( $36^{\circ}\text{F}$  to  $46^{\circ}\text{F}$ ) in the upright position.
- Do not refreeze.
- Do not shake.
- Do not place back in the refrigerator once brought to room temperature.
- Follow local guidelines on handling of biological waste.

## 17 PATIENT COUNSELING INFORMATION

### Inform caregivers that:

- ELEVIDYS can increase certain liver enzyme levels and cause acute serious liver injury. Patients will receive oral corticosteroid medication before and after infusion with ELEVIDYS. Weekly blood tests will be required to monitor liver enzyme levels for 3 months after treatment. Contact a healthcare provider immediately if the patient's skin and/or whites of the eyes appear yellowish, or if the patient misses a dose of corticosteroid or vomits it up [see *Warnings and Precautions* (5.1)].
- Immune-mediated myositis (an immune response affecting muscles) was observed in patients with a deletion mutation in the *DMD* gene that is contraindicated. Contact a physician immediately if the patient experiences any unexplained increased muscle pain, tenderness, or weakness, including difficulty swallowing, difficulty breathing or difficulty speaking, as these may be symptoms of myositis [see *Warnings and Precautions* (5.2)].
- Myocarditis (inflammation of the heart) has been observed within days following ELEVIDYS infusion. Weekly monitoring of troponin-I for the first month after treatment is required. Contact a healthcare provider immediately if the patient begins to experience chest pain and/or shortness of breath [see *Warnings and Precautions* (5.3)].
- Patient's immunizations should be up-to-date with current immunization guidelines prior to initiation of the corticosteroid regimen required before ELEVIDYS infusion. Vaccinations should be completed at least 4 weeks prior to initiation of the corticosteroid regimen [see *Drug Interactions* (7)].
- Due to the concomitant administration of corticosteroids, an infection (e.g., cold, flu, gastroenteritis, otitis media, bronchiolitis, etc.) before or after ELEVIDYS infusion could lead to more serious complications. Contact a healthcare provider immediately if symptoms suggestive of infection are observed (e.g., coughing, wheezing, sneezing, runny nose, sore throat, or fever).
- Vector shedding of ELEVIDYS occurs primarily through body waste. Practice proper hand hygiene, such as hand washing, when coming into direct contact with patient body waste. Place potentially contaminated materials that may have the patient's bodily fluids/waste in a sealable bag and dispose into regular trash. These precautions should be followed for one month after ELEVIDYS infusion.

Manufactured for: Sarepta Therapeutics, Inc.  
Cambridge, MA 02142 USA  
U.S. license number 2308



**HIGHLIGHTS OF PRESCRIBING INFORMATION**

These highlights do not include all the information needed to use VYONDYS 53 safely and effectively. See full prescribing information for VYONDYS 53.

**VYONDYS 53 (golodirsen) injection, for intravenous use**  
Initial U.S. Approval: 2019

**RECENT MAJOR CHANGES**

Dosage and Administration (2.1, 2.2, 2.3, 2.4) 2/2021  
Warnings and Precautions (5.2) 2/2021

**INDICATIONS AND USAGE**

VYONDYS 53 is an antisense oligonucleotide indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the *DMD* gene that is amenable to exon 53 skipping. This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with VYONDYS 53. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials. (1)

**DOSAGE AND ADMINISTRATION**

- Serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio should be measured before starting VYONDYS 53 (2.1)
- 30 milligrams per kilogram once weekly (2.2)
- Administer as an intravenous infusion over 35 to 60 minutes via an in-line 0.2 micron filter (2.2, 2.4)
- Dilution required prior to administration (2.3)

**DOSAGE FORMS AND STRENGTHS**

Injection: 100 mg/2 mL (50 mg/mL) in a single-dose vial (3)

**CONTRAINDICATIONS**

None (4)

**WARNINGS AND PRECAUTIONS**

- Hypersensitivity Reactions:** Hypersensitivity reactions, including rash, pyrexia, pruritus, urticaria, dermatitis, and skin exfoliation have occurred in patients who were treated with VYONDYS 53. If a hypersensitivity reaction occurs, institute appropriate medical treatment and consider slowing the infusion or interrupting the VYONDYS 53 therapy. (2.3, 5.1)
- Kidney Toxicity:** Based on animal data, may cause kidney toxicity. Kidney function should be monitored; creatinine may not be a reliable measure of renal function in DMD patients. (5.2, 13.2)

**ADVERSE REACTIONS**

The most common adverse reactions (incidence  $\geq 20\%$  and higher than placebo) were headache, pyrexia, fall, abdominal pain, nasopharyngitis, cough, vomiting, and nausea. (6.1)

To report SUSPECTED ADVERSE REACTIONS, contact Sarepta Therapeutics, Inc. at 1-888-SAREPTA (1-888-727-3782) or FDA at 1-800-FDA-1088 or [www.fda.gov/medwatch](http://www.fda.gov/medwatch).

See 17 for PATIENT COUNSELING INFORMATION

Revised: 2/2021

**FULL PRESCRIBING INFORMATION: CONTENTS\***

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## FULL PRESCRIBING INFORMATION

### 1 INDICATIONS AND USAGE

VYONDYS 53 is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the *DMD* gene that is amenable to exon 53 skipping. This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with VYONDYS 53 [see *Clinical Studies* (14)]. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

### 2 DOSAGE AND ADMINISTRATION

#### 2.1 Monitoring to Assess Safety

Serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio should be measured before starting VYONDYS 53. Consider measurement of glomerular filtration rate prior to initiation of VYONDYS 53. Monitoring for kidney toxicity during treatment is recommended. Obtain the urine samples prior to infusion of VYONDYS 53 or at least 48 hours after the most recent infusion [see *Warnings and Precautions* (5.2)].

#### 2.2 Dosing Information

The recommended dosage of VYONDYS 53 is 30 milligrams per kilogram administered once weekly as a 35 to 60-minute intravenous infusion via an in-line 0.2 micron filter.

If a dose of VYONDYS 53 is missed, it may be administered as soon as possible after the scheduled dose.

#### 2.3 Preparation Instructions

VYONDYS 53 is supplied in single-dose vials as a preservative-free concentrated solution that requires dilution prior to administration. Parenteral drug products should be inspected visually for particulate matter and discoloration prior to administration, whenever solution and container permit. Use aseptic technique.

- a. Calculate the total dose of VYONDYS 53 to be administered based on the patient's weight and the recommended dose of 30 milligrams per kilogram. Determine the volume of VYONDYS 53 needed and the correct number of vials to supply the full calculated dose.
- b. Allow the vials to warm to room temperature. Mix the contents of each vial by gently inverting 2 or 3 times. Do not shake.
- c. Visually inspect each vial of VYONDYS 53. The solution is a clear to slightly opalescent, colorless liquid, and may contain trace amounts of small, white to off-white amorphous particles. Do not use if the solution in the vials is cloudy, discolored or



contains extraneous particulate matter other than trace amounts of small, white to off-white amorphous particles.

- d. With a syringe fitted with a 21-gauge or smaller bore non-coring needle, withdraw the calculated volume of VYONDYS 53 from the appropriate number of vials.
- e. Dilute the withdrawn VYONDYS 53 in 0.9% Sodium Chloride Injection, USP, to make a total volume of 100 to 150 mL. Gently invert 2 to 3 times to mix. Do not shake. Visually inspect the diluted solution. Do not use if the solution is cloudy, discolored or contains extraneous particulate matter other than trace amounts of small, white to off-white amorphous particles.
- f. Administer the diluted solution via an in-line 0.2 micron filter.
- g. VYONDYS 53 contains no preservatives and should be administered immediately after dilution. Complete infusion of diluted VYONDYS 53 within 4 hours of dilution. If immediate use is not possible, the diluted product may be stored for up to 24 hours at 2°C to 8°C (36°F to 46°F). Do not freeze. Discard unused VYONDYS 53.

## 2.4 Administration Instructions

Application of a topical anesthetic cream to the infusion site prior to administration of VYONDYS 53 may be considered.

VYONDYS 53 is administered via intravenous infusion. Flush the intravenous access line with 0.9% Sodium Chloride Injection, USP, prior to and after infusion.

Infuse the diluted VYONDYS 53 over 35 to 60 minutes via an in-line 0.2 micron filter. Do not mix other medications with VYONDYS 53 or infuse other medications concomitantly via the same intravenous access line with VYONDYS 53.

If a hypersensitivity reaction occurs, consider slowing the infusion or interrupting the VYONDYS 53 therapy [*see Warnings and Precautions (5.1) and Adverse Reactions (6.1)*].

## 3 DOSAGE FORMS AND STRENGTHS

VYONDYS 53 is a clear to slightly opalescent, colorless liquid, and may contain trace amounts of small, white to off-white amorphous particles, and available as:

- Injection: 100 mg/2 mL (50 mg/mL) solution in a single-dose vial

## 4 CONTRAINDICATIONS

None.

## **5 WARNINGS AND PRECAUTIONS**

### **5.1 Hypersensitivity Reactions**

Hypersensitivity reactions, including rash, pyrexia, pruritus, urticaria, dermatitis, and skin exfoliation have occurred in VYONDYS 53-treated patients, some requiring treatment. If a hypersensitivity reaction occurs, institute appropriate medical treatment and consider slowing the infusion or interrupting the VYONDYS 53 therapy [see *Dosage and Administration* (2.4)].

### **5.2 Kidney Toxicity**

Kidney toxicity was observed in animals who received golodirsen [see *Use in Specific Populations* (8.4)]. Although kidney toxicity was not observed in the clinical studies with VYONDYS 53, the clinical experience with VYONDYS 53 is limited, and kidney toxicity, including potentially fatal glomerulonephritis, has been observed after administration of some antisense oligonucleotides. Kidney function should be monitored in patients taking VYONDYS 53. Because of the effect of reduced skeletal muscle mass on creatinine measurements, creatinine may not be a reliable measure of kidney function in DMD patients. Serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio should be measured before starting VYONDYS 53. Consider also measuring glomerular filtration rate using an exogenous filtration marker before starting VYONDYS 53. During treatment, monitor urine dipstick every month, and serum cystatin C and urine protein-to-creatinine ratio every three months. Only urine expected to be free of excreted VYONDYS 53 should be used for monitoring of urine protein. Urine obtained on the day of VYONDYS 53 infusion prior to the infusion, or urine obtained at least 48 hours after the most recent infusion, may be used. Alternatively, use a laboratory test that does not use the reagent pyrogallol red, as this reagent has the potential to cross react with any VYONDYS 53 that is excreted in the urine and thus lead to a false positive result for urine protein.

If a persistent increase in serum cystatin C or proteinuria is detected, refer to a pediatric nephrologist for further evaluation.

## **6 ADVERSE REACTIONS**

The following serious adverse reactions are described below and elsewhere in the labeling:

- Hypersensitivity Reactions [see *Warnings and Precautions* (5.1)]

### **6.1 Clinical Trials Experience**

Because clinical trials are conducted under widely varying conditions, adverse reaction rates observed in the clinical trials of a drug cannot be directly compared to rates in the clinical trials of another drug and may not reflect the rates observed in practice.

In the VYONDYS 53 clinical development program, 58 patients received at least one intravenous dose of VYONDYS 53, ranging between 4 mg/kg (0.13 times the recommended dosage) and 30 mg/kg (the recommended dosage). All patients were male and had genetically confirmed Duchenne muscular dystrophy. Age at study entry was 6 to 13 years. Most (86%) patients were Caucasian.

VYONDYS 53 was studied in 2 double-blind, placebo-controlled studies.

In Study 1 Part 1, patients were randomized to receive once-weekly intravenous infusions of VYONDYS 53 (n=8) in four increasing dose levels from 4 mg/kg to 30 mg/kg or placebo (n=4), for at least 2 weeks at each level. All patients who participated in Study 1 Part 1 (n=12) were continued into Study 1 Part 2, an open-label extension, during which they received VYONDYS 53 at a dose of 30 mg/kg IV once weekly [see *Clinical Studies* (14)].

In Study 2, patients received VYONDYS 53 (n=33) 30 mg/kg or placebo (n=17) IV once weekly for up to 96 weeks, after which all patients received VYONDYS 53 at a dose of 30 mg/kg.

Adverse reactions observed in at least 20% of treated patients in the placebo-controlled sections of Studies 1 and 2 are shown in [Table 1](#).

**Table 1: Adverse Reactions That Occurred in At Least 20% of VYONDYS 53-Treated Patients and at a Rate Greater than Placebo in Studies 1 and 2**

Adverse Reaction	VYONDYS 53 (N = 41) %	Placebo (N = 21) %
Headache	41	10
Pyrexia	41	14
Fall	29	19
Abdominal pain	27	10
Nasopharyngitis	27	14
Cough	27	19
Vomiting	27	19
Nausea	20	10

Other adverse reactions that occurred at a frequency greater than 5% of VYONDYS 53-treated patients and at a greater frequency than placebo were: administration site pain, back pain, pain, diarrhea, dizziness, ligament sprain, contusion, influenza, oropharyngeal pain, rhinitis, skin abrasion, ear infection, seasonal allergy, tachycardia, catheter site related reaction, constipation, and fracture.

Hypersensitivity reactions have occurred in patients treated with VYONDYS 53 [see *Warnings and Precautions* (5.1)].

## **8 USE IN SPECIFIC POPULATIONS**

### **8.1 Pregnancy**

#### Risk Summary

There are no human or animal data available to assess the use of VYONDYS 53 during pregnancy. In the U.S. general population, major birth defects occur in 2 to 4% and miscarriage occurs in 15 to 20% of clinically recognized pregnancies.

### **8.2 Lactation**

#### Risk Summary

There are no human or animal data to assess the effect of VYONDYS 53 on milk production, the presence of golodirsen in milk, or the effects of VYONDYS 53 on the breastfed infant.

The developmental and health benefits of breastfeeding should be considered along with the mother's clinical need for VYONDYS 53 and any potential adverse effects on the breastfed infant from VYONDYS 53 or from the underlying maternal condition.

### **8.4 Pediatric Use**

VYONDYS 53 is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the *DMD* gene that is amenable to exon 53 skipping, including pediatric patients [*see Clinical Studies (14)*].

Intravenous administration of golodirsen (0, 100, 300, or 900 mg/kg) to juvenile male rats once weekly for 10 weeks (postnatal days 14 to 77) did not result in postnatal developmental (e.g., neurobehavioral, immune function, or male reproductive) toxicity. However, at the highest dose tested (900 mg/kg/week), golodirsen resulted in the death of animals because of renal impairment or failure. In surviving animals (including one animal at the lowest dose tested), there was a dose-dependent increase in the incidence and severity of renal tubular effects (including degeneration/regeneration, fibrosis, vacuolation, and dilatation), which correlated with changes in clinical pathology parameters, reflecting a dose-dependent impairment of renal function. In addition, decreases in bone area, mineral content, and mineral density were observed at the highest dose tested (900 mg/kg/week) but with no effect on bone growth. A no-effect dose for renal toxicity was not identified; the lowest dose tested (100 mg/kg/week) was associated with plasma exposures (AUC) approximately 2.5 times that in humans at the recommended human dose of 30 mg/kg/week.

### **8.5 Geriatric Use**

DMD is largely a disease of children and young adults; therefore, there is no geriatric experience with VYONDYS 53.

## 8.6 Patients with Renal Impairment

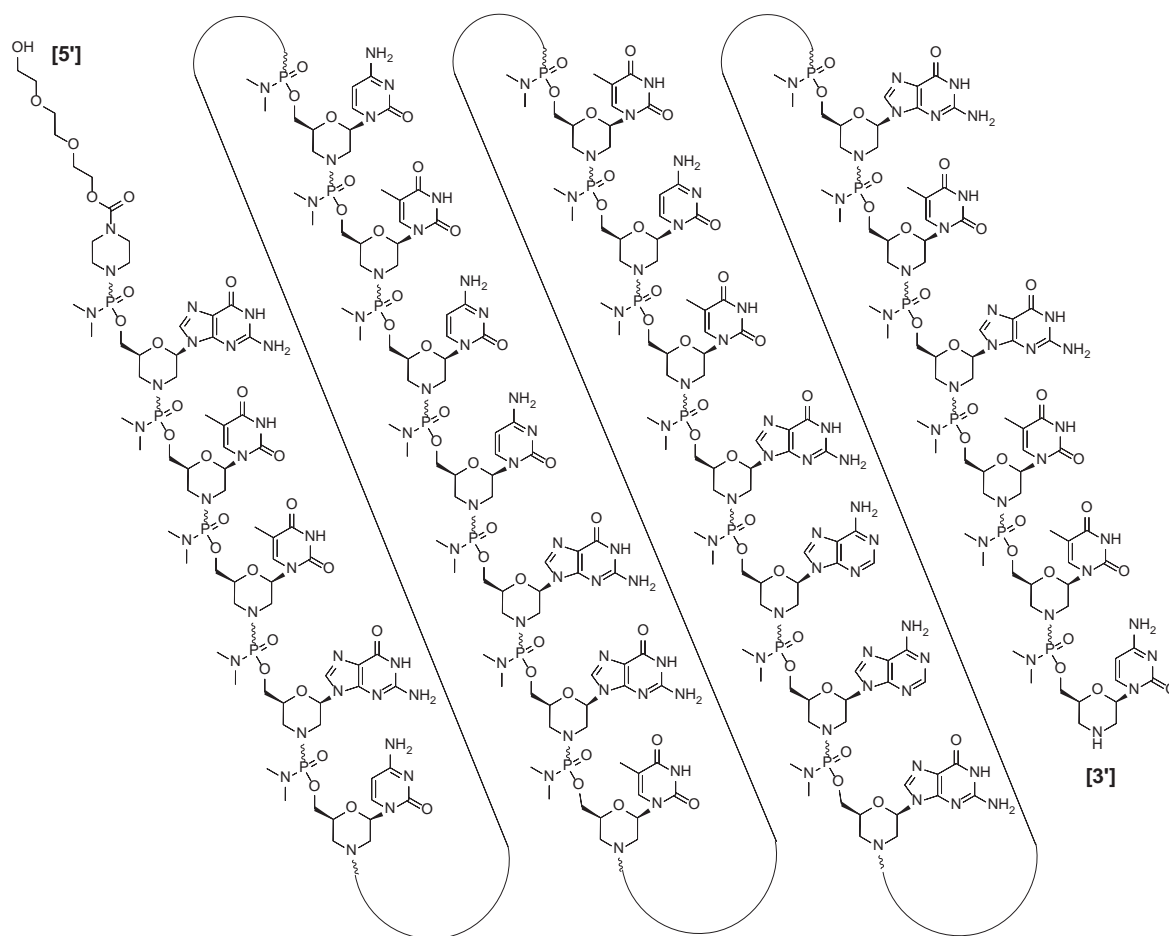
Renal clearance of golodirsen is reduced in non-DMD adults with renal impairment, based on estimated glomerular filtration rate calculated using the Modification of Diet and Renal Disease (MDRD) equation [see *Clinical Pharmacology* (12.3)]. However, because of the effect of reduced skeletal muscle mass on creatinine measurements in DMD patients, no specific dosage adjustment can be recommended for DMD patients with renal impairment based on estimated glomerular filtration rate. Patients with known renal function impairment should be closely monitored during treatment with VYONDYS 53.

## 11 DESCRIPTION

VYONDYS 53 (golodirsen) injection is a sterile, aqueous, preservative-free, concentrated solution for dilution prior to intravenous administration. VYONDYS 53 is a clear to slightly opalescent, colorless liquid, and may contain trace amounts of small, white to off-white amorphous particles. VYONDYS 53 is supplied in single-dose vials containing 100 mg golodirsen (50 mg/mL). VYONDYS 53 is formulated as an isotonic phosphate buffered saline solution with an osmolality of 260 to 320 mOSM and a pH of 7.5. Each milliliter of VYONDYS 53 contains: 50 mg golodirsen; 0.2 mg potassium chloride; 0.2 mg potassium phosphate monobasic; 8 mg sodium chloride; and 1.14 mg sodium phosphate dibasic, anhydrous, in water for injection. The product may contain hydrochloric acid or sodium hydroxide to adjust pH.

Golodirsen is an antisense oligonucleotide of the phosphorodiamidate morpholino oligomer (PMO) subclass. PMOs are synthetic molecules in which the five-membered ribofuranosyl rings found in natural DNA and RNA are replaced by a six-membered morpholino ring. Each morpholino ring is linked through an uncharged phosphorodiamidate moiety rather than the negatively charged phosphate linkage that is present in natural DNA and RNA. Each phosphorodiamidate morpholino subunit contains one of the heterocyclic bases found in DNA (adenine, cytosine, guanine, or thymine). Golodirsen contains 25 linked subunits. The sequence of bases from the 5' end to 3' end is GTTGCCTCCGTTCTGAAGGTGTTC. The molecular formula of golodirsen is  $C_{305}H_{481}N_{138}O_{112}P_{25}$  and the molecular weight is 8647.28 daltons.

The structure of golodirsen is:



## 12 CLINICAL PHARMACOLOGY

### 12.1 Mechanism of Action

Golodirsen is designed to bind to exon 53 of dystrophin pre-mRNA resulting in exclusion of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 53 skipping. Exon 53 skipping is intended to allow for production of an internally truncated dystrophin protein in patients with genetic mutations that are amenable to exon 53 skipping [see *Clinical Studies* (14)].

### 12.2 Pharmacodynamics

After treatment with VYONDYS 53, all patients evaluated (n=25) in Study 1 Part 2 [see *Clinical Studies* (14)] had an increase in skipping of exon 53 demonstrated by reverse transcription polymerase chain reaction (RT-PCR), compared to baseline.

In Study 1 Part 2 [see *Clinical Studies* (14)], dystrophin levels as assessed by the Sarepta western blot assay increased from 0.10% (SD 0.07) of normal at baseline to 1.02% (SD 1.03) of normal after 48 weeks of treatment with VYONDYS 53. The mean change from baseline in

dystrophin after 48 weeks of treatment with VYONDYS 53 was 0.92% (SD 1.01) of normal levels ( $p < 0.001$ ); the median change from baseline was 0.88%. This increase in dystrophin protein expression positively correlated with the level of exon skipping. Dystrophin levels assessed by western blot can be meaningfully influenced by differences in sample processing, analytical technique, reference materials, and quantitation methodologies. Therefore, comparing dystrophin results from different assay protocols will require a standardized reference material and additional bridging studies.

Correct localization of truncated dystrophin to the sarcolemma in muscle fibers of patients treated with golodirsen was demonstrated by immunofluorescence staining.

### **12.3 Pharmacokinetics**

The pharmacokinetics of golodirsen was evaluated in DMD patients following administration of intravenous doses ranging from 4 mg/kg/week to 30 mg/kg/week (i.e., recommended dosage). Golodirsen exposure increased proportionally with dose, with minimal accumulation with once-weekly dosing. Inter-subject variability (as %CV) for  $C_{\max}$  and AUC ranged from 38% to 72%, and 34% to 44%, respectively.

#### Distribution

Steady-state volume of distribution was similar between DMD patients and healthy subjects. The mean golodirsen steady-state volume of distribution was 668 mL/kg (%CV=32.3) at a dose of 30 mg/kg. Golodirsen plasma protein binding ranged from 33% to 39% and is not concentration dependent.

#### Elimination

Golodirsen elimination half-life (SD) was 3.4 (0.6) hours, and plasma clearance was 346 mL/hr/kg at the 30 mg/kg dose.

#### Metabolism

Golodirsen is metabolically stable. No metabolites were detected in plasma or urine.

#### Excretion

Golodirsen is mostly excreted unchanged in the urine. The elimination half-life ( $t_{1/2}$ ) was 3.4 hours.

#### Specific Populations

##### *Age:*

The pharmacokinetics of golodirsen have been evaluated in male pediatric DMD patients. There is no experience with the use of VYONDYS 53 in DMD patients 65 years of age or older.

##### *Sex:*

Sex effects have not been evaluated; VYONDYS 53 has not been studied in female patients.

##### *Race:*



The potential impact of race is not known because 92% of the patients in studies were Caucasians.

*Patients with Renal Impairment:*

The effect of renal impairment on the pharmacokinetics of golodirsen was evaluated in non-DMD subjects aged 41 to 65 years with Stage 2 chronic kidney disease (CKD) (n=8, estimated glomerular filtration rate (eGFR)  $\geq 60$  and  $< 90$  mL/min/1.73 m<sup>2</sup>) or Stage 3 CKD (n=8, eGFR  $\geq 30$  and  $< 60$  mL/min/1.73 m<sup>2</sup>) and matched healthy subjects (n=8, eGFR  $\geq 90$  mL/min/1.73 m<sup>2</sup>). Subjects received a single 30 mg/kg IV dose of golodirsen.

In subjects with Stage 2 or Stage 3 CKD, exposure (AUC) increased approximately 1.2-fold and 1.9-fold, respectively. There was no change in the C<sub>max</sub> in subjects with Stage 2 CKD; in subjects with Stage 3 CKD, there was a 1.2-fold increase in C<sub>max</sub> compared with subjects with normal renal function. The effect of Stage 4 or Stage 5 CKD on golodirsen pharmacokinetics and safety has not been studied.

Estimated GFR values derived from MDRD equations and the threshold definitions for various CKD stages in otherwise healthy adults would not be generalizable to pediatric patients with DMD. Therefore, no specific dosage adjustment can be recommended for patients with renal impairment [see *Use in Specific Populations* (8.6)].

*Patients with Hepatic Impairment:*

VYONDYS 53 has not been studied in patients with hepatic impairment.

Drug Interaction Studies

Golodirsen did not inhibit CYP1A2, CYP2B6, CYP2C8, CYP2C9, CYP2C19, CYP2D6, or CYP3A4/5 *in vitro*. Golodirsen was a weak inducer of CYP1A2 and did not induce CYP2B6 or CYP3A4. Golodirsen was not metabolized by human hepatic microsomes and was not a substrate or strong inhibitor of any of the key human drug transporters tested (OAT1, OAT3, OCT2, OATP1B1, MATE1, P-gp, BCRP, and MRP2, OATP1B3 and MATE2-K). Based on *in vitro* data, golodirsen has a low potential for drug-drug interactions in humans.

## **13 NONCLINICAL TOXICOLOGY**

### **13.1 Carcinogenesis, Mutagenesis, Impairment of Fertility**

Carcinogenesis

Carcinogenicity studies have not been conducted with golodirsen.

Mutagenesis

Golodirsen was negative in *in vitro* (bacterial reverse mutation and chromosomal aberration in CHO cells) and *in vivo* (mouse bone marrow micronucleus) assays.

Impairment of Fertility

Fertility studies in animals were not conducted with golodirsen. No effects of golodirsen on the male reproductive system were observed following weekly subcutaneous administration (0, 120,



300, or 600 mg/kg to male mice or weekly intravenous administration (0, 80, 200, or 400 mg/kg) to male monkeys. Plasma exposure (AUC) at the highest doses tested in mouse and monkey are approximately 10 and 45 times that in humans at the recommended weekly intravenous dose of 30 mg/kg.

### **13.2 Animal Toxicology and/or Pharmacology**

Kidney toxicity was observed in studies in male mice and rats; findings in urinary bladder were observed in male mice.

In male mice, golodirsen was administered weekly for 12 weeks by intravenous injection (0, 12, 120, or 960 mg/kg) or for 26 weeks by subcutaneous injection (0, 120, 300, or 600 mg/kg). In the 12-week study, microscopic findings in kidney (tubular dilatation, basophilic or eosinophilic casts, vacuolation), correlated with increases in serum markers of renal function (e.g., urea nitrogen, creatinine), were observed primarily at the highest dose tested; hypertrophy of the transitional epithelium of the ureter or urinary bladder was observed at all doses. In the 26-week study, renal tubular degeneration and degeneration of the transitional epithelium of the urinary bladder were observed at all doses.

In male rats, intravenous administration of golodirsen (0, 60, 100, 300, or 600 mg/kg) weekly for 13 weeks resulted in tubular degeneration at all but the lowest dose tested; at the high dose, the microscopic changes were accompanied by increases in serum urea nitrogen.

In male monkeys, intravenous administration of golodirsen (0, 80, 200, or 400 mg/kg) weekly for 39 weeks resulted in microscopic changes in kidney (basophilia, dilatation, or mononuclear cell infiltration) at all doses, which correlated with increases in serum markers of renal function (urea nitrogen, creatinine) at the highest dose tested.

## **14 CLINICAL STUDIES**

The effect of VYONDYS 53 on dystrophin production was evaluated in one study in DMD patients with a confirmed mutation of the *DMD* gene that is amenable to exon 53 skipping (Study 1; NCT02310906).

Study 1 Part 1 was a double-blind, placebo-controlled, dose-titration study in 12 DMD patients. Patients were randomized 2:1 to receive VYONDYS 53 or matching placebo. VYONDYS 53-treated patients received four escalating dose levels, ranging from 4 mg/kg/week (less than the recommended dosage) to 30 mg/kg/week, by intravenous infusion for 2 weeks at each dose level.

Study 1 Part 2 was a 168-week, open-label study assessing the efficacy and safety of VYONDYS 53 at a dose of 30 mg/kg/week in the 12 patients enrolled in Part 1, plus 13 additional treatment-naïve patients with DMD amenable to exon 53 skipping. At study entry (either in Part 1 or Part 2), patients had a median age of 8 years and were on a stable dose of corticosteroids for at least 6 months. Efficacy was assessed based on change from baseline in the dystrophin protein level (measured as % of the dystrophin level in healthy subjects, i.e., % of normal) at Week 48 of Part 2. Muscle biopsies were obtained at baseline prior to treatment and at Week 48 of Part 2 in all VYONDYS 53-treated patients (n=25), and were analyzed for dystrophin protein level by Sarepta western blot. Mean dystrophin levels increased from 0.10% (SD 0.07) of normal at

baseline to 1.02% (SD 1.03) of normal by Week 48 of Study 1 Part 2, with a mean change in dystrophin of 0.92% (SD 1.01) of normal levels ( $p < 0.001$ ); the median change from baseline was 0.88%.

Individual patient dystrophin levels from Study 1 are shown in [Table 2](#).

**Table 2: Dystrophin Expression Sarepta Western Blot by Individual Patient From Study 1**

Patient Number	Sarepta Western Blot % Normal Dystrophin			Patient number	Sarepta Western Blot % Normal Dystrophin		
	Baseline	Part 2 Week 48	Change from baseline		Baseline	Part 2 Week 48	Change from baseline
1	0.08	0.09	0.01	14	0.22	0.28	0.06
2	0.11	0.11	0.01	15	0.14	0.21	0.07
3	0.21	0.22	0.01	16	0.05	0.42	0.37
4	0.05	0.12	0.08	17	0.07	1.03	0.97
5	0.03	0.12	0.09	18	0.02	1.57	1.55
6	0.06	0.14	0.09	19	0.12	1.17	1.05
7	0.12	0.37	0.25	20	0.03	1.72	1.69
8	0.11	1.06	0.95	21	0.11	1.77	1.66
9	0.06	0.54	0.48	22	0.31	4.30	3.99
10	0.05	0.97	0.92	23	0.11	0.36	0.25
11	0.06	1.55	1.49	24	0.03	0.91	0.88
12	0.07	1.91	1.84	25	0.07	1.29	1.22
13	0.10	3.25	3.15				

## 16 HOW SUPPLIED/STORAGE AND HANDLING

### 16.1 How Supplied

VYONDYS 53 injection is supplied in single dose vials. The solution is a clear to slightly opalescent, colorless liquid, and may contain trace amounts of small, white to off-white amorphous particles.

- Single-dose vials containing 100 mg/2mL (50 mg/mL) NDC 60923-465-02

### 16.2 Storage and Handling

Store VYONDYS 53 at 2°C to 8°C (36°F to 46°F). Do not freeze. Store in original carton until ready for use to protect from light.

## 17 PATIENT COUNSELING INFORMATION

### Hypersensitivity Reactions

Advise patients and/or caregivers that hypersensitivity reactions, including rash, pyrexia, pruritus, urticaria, dermatitis, and skin exfoliation have occurred in patients who were treated with VYONDYS 53. Instruct them to seek immediate medical care should they experience signs and symptoms of hypersensitivity [*see Warnings and Precautions (5.1)*].

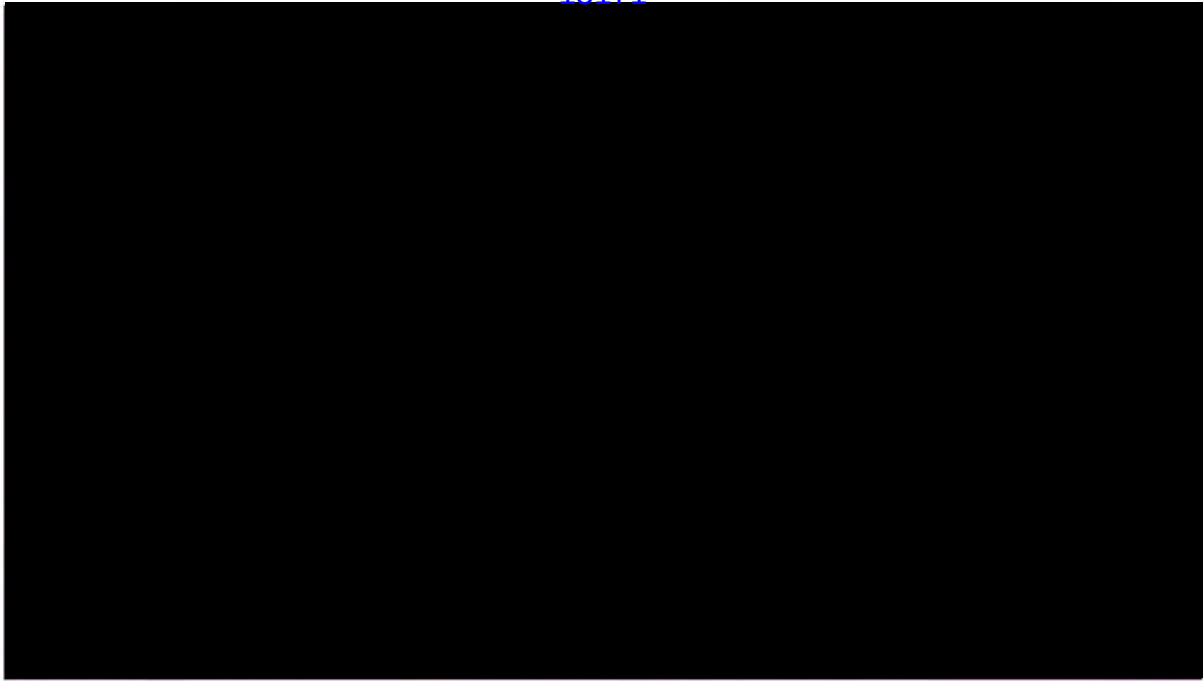
### Kidney Toxicity

Inform patients nephrotoxicity has occurred with drugs similar to VYONDYS 53. Advise patients of the importance of monitoring for kidney toxicity by their healthcare providers during treatment with VYONDYS 53 [*see Warnings and Precautions (5.2)*].

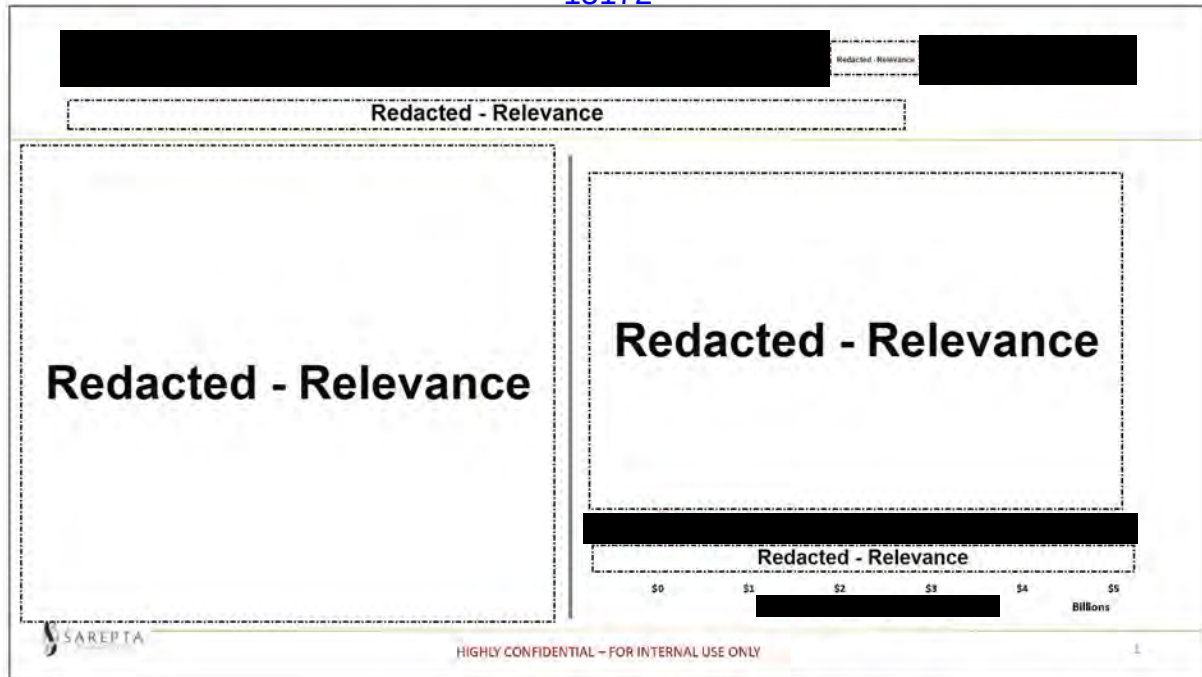
Manufactured for:  
Sarepta Therapeutics, Inc.  
Cambridge, MA 02142 USA

SAREPTA, SAREPTA THERAPEUTICS, and VYONDYS are trademarks of Sarepta Therapeutics, Inc. registered in the U.S. Patent and Trademark Office and may be registered in various other jurisdictions. VYONDYS 53, and the Vyondys 53 logo are trademarks of Sarepta Therapeutics, Inc.



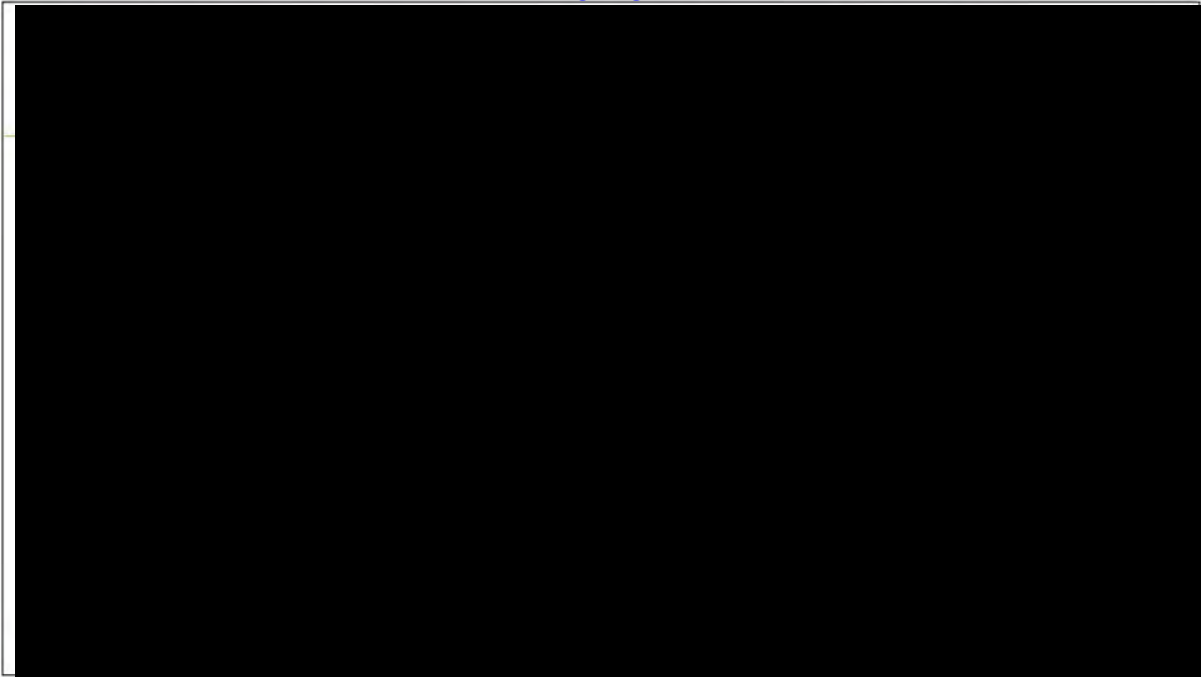


Notes view: 0




**Redacted - Relevance**

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• Redacted - Relevance

• Redacted - Relevance

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**Redacted - Relevance**

 EU not included

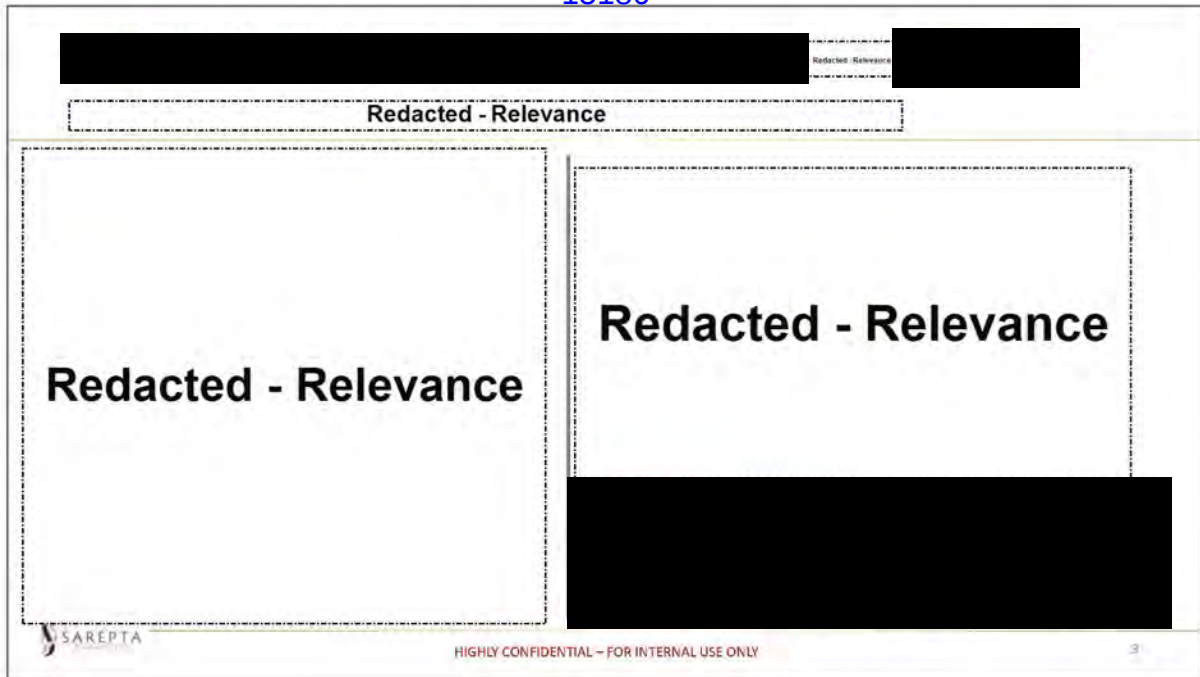
HIGHLY CONFIDENTIAL - FOR INTERNAL USE ONLY

2

**Redacted - Relevance**

**Redacted - Relevance**

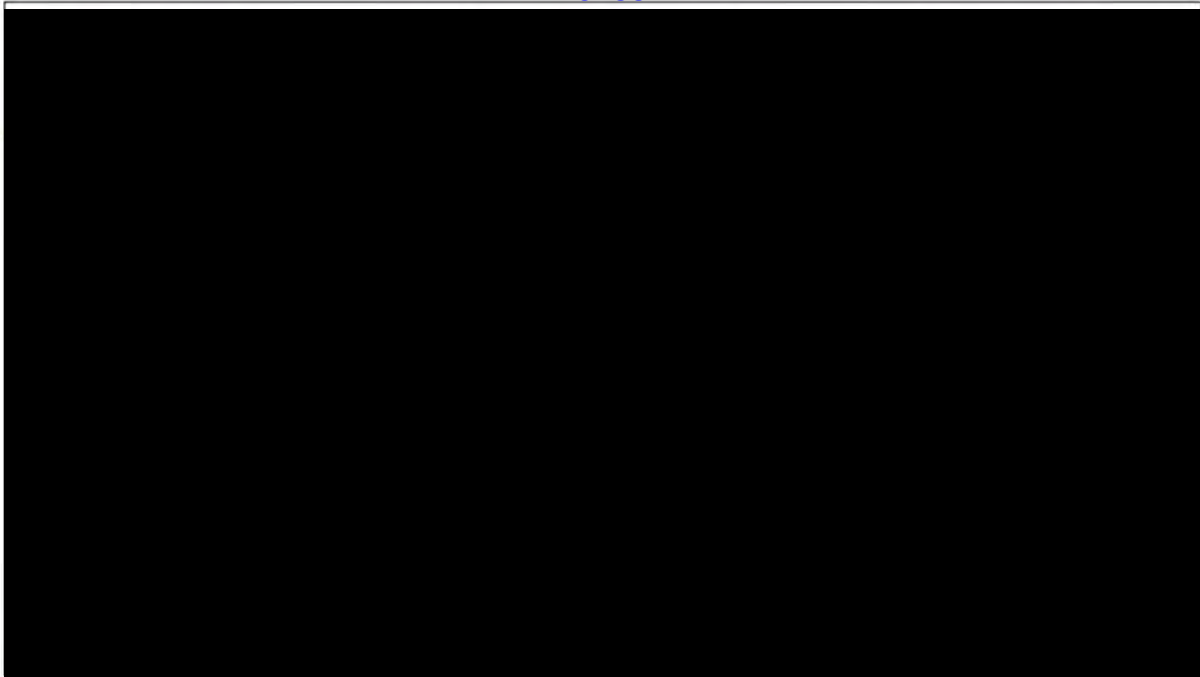
**Redacted - Relevance**



**Redacted - Relevance**

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**Redacted - Relevance**

**Redacted - Relevance**

IN THE UNITED STATES DISTRICT COURT  
FOR THE DISTRICT OF DELAWARE

NIPPON SHINYAKU CO., LTD.,	)	
	)	
Plaintiff,	)	
	)	
v.	)	C.A. No. 21-1015 (MN)
	)	
SAREPTA THERAPEUTICS, INC.,	)	
	)	
Defendant.	)	
<hr/>		
SAREPTA THERAPEUTICS, INC.,	)	
	)	
Defendant and Counter-Plaintiff,	)	
	)	
v.	)	
	)	
NIPPON SHINYAKU CO., LTD.	)	
and NS PHARMA, INC.	)	
	)	
Plaintiff and Counter-	)	
Defendants.	)	

**SAREPTA’S RESPONSES AND OBJECTIONS TO NS’S FIRST SET OF  
REQUESTS FOR PRODUCTION TO SAREPTA (NOS. 1-149)**

Pursuant to Rules 26 and 34 of the Federal Rules of Civil Procedure, Defendant/Counter-Plaintiff Sarepta Therapeutics, Inc. (“Sarepta”), through the undersigned counsel, hereby objects and responds to Plaintiff/Counter-Defendant Nippon Shinyaku Co., Ltd.’s (“Nippon Shinyaku”) and Counter-Defendant NS Pharma, Inc.’s (“NS Pharma”) (collectively, “NS”) First Set of Requests for Production of Documents (Nos. 1-149) served on March 11, 2022.

**GENERAL OBJECTIONS**

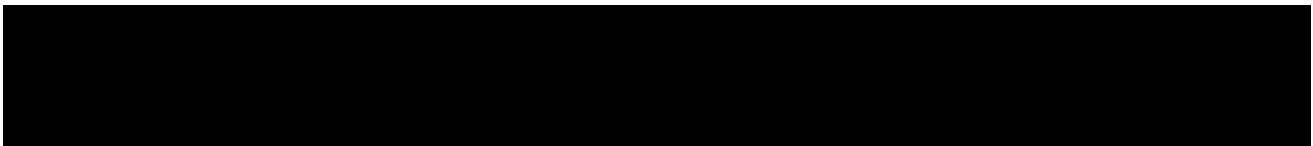
Sarepta makes the following general objections, which are hereby incorporated in their entirety in response to each individual request, even if not separately stated therein:

**RESPONSE**

Sarepta incorporates its General Objections here as if set forth in full. Sarepta further objects to this request to the extent it seeks the production of documents or information protected by the attorney-client privilege, the work-product doctrine, or any other privilege or immunity, and/or claim of third-party confidentiality. Sarepta also objects to this request as overly broad and unduly burdensome to the extent it seeks “[a]ll” documents because it is not limited to documents and information relevant to, and reasonably and proportionally directed to, the claims and defenses at issue in this case. Sarepta further objects to this request as vague and ambiguous as to what it seeks (“infringement of any NS Patent by the Accused NS Products”). Sarepta objects to this request to the extent it seeks documents that are not in the possession, custody, or control of Sarepta. Sarepta objects to this request to the extent it seeks documents that are publicly available, readily identifiable, and equally accessible to NS. Sarepta objects to this request as premature because the Court has not entered a scheduling order in this litigation.

Subject to and without waiving these specific objections and the General Objections, Sarepta will produce relevant, nonprivileged documents relating to damages for NS’s infringement of the UWA Patents, in accordance with the Case Schedule, Local Rules, and Federal Rules of Civil Procedure. Sarepta reserves the right to supplement this response.

**REQUEST NO. 101:**



**RESPONSE**

Sarepta incorporates its General Objections here as if set forth in full. Sarepta further objects to this request to the extent it seeks the production of documents or information protected



by the attorney-client privilege, the work-product doctrine, or any other privilege or immunity, and/or claim of third-party confidentiality. Sarepta also objects to this request as overly broad and unduly burdensome to the extent it seeks “[a]ll” documents because it is not limited to documents and information relevant to, and reasonably and proportionally directed to, the claims and defenses at issue in this case. Sarepta further objects to this request as vague and ambiguous as the terms “the field” and “technology related to treatments for Duchenne’s Muscular Dystrophy or antisense oligonucleotides” are not defined. Sarepta further objects to this request to the extent it seeks information and documents that are not relevant or reasonably and proportionally directed to the claims and defenses at issue in this case. Sarepta objects to this request to the extent it seeks documents that are publicly available, readily identifiable, and equally accessible to NS.

Subject to and without waiving these specific objections and the General Objections,

[REDACTED]

[REDACTED] to the extent they exist, have not already been produced, and are located within Sarepta’s possession, custody, or control after a reasonable search consistent with the forthcoming ESI Protocol and in accordance with the Case Schedule, Local Rules, and Federal Rules of Civil Procedure. Sarepta reserves the right to supplement this response.

**REQUEST NO. 102:**

All Documents relating to the advantages of, disadvantages of, results achieved by, or suitability of treatments for Duchenne Muscular Dystrophy other than the Sarepta Patent-Practicing Products and Accused NS Products.

**RESPONSE**

Sarepta incorporates its General Objections here as if set forth in full. Sarepta further objects to this request to the extent it seeks the production of documents or information protected by the attorney-client privilege, the work-product doctrine, or any other privilege or immunity,

and/or claim of third-party confidentiality. Sarepta also objects to this request as overly broad and unduly burdensome to the extent it seeks “[a]ll” documents because it is not limited to documents and information relevant to, and reasonably and proportionally directed to, the claims and defenses at issue in this case. Sarepta further objects to this request to the extent it seeks information and documents that are not relevant or reasonably and proportionally directed to the claims and defenses at issue in this case. Sarepta objects to this request to the extent it seeks documents that are publicly available, readily identifiable, and equally accessible to NS. Sarepta further objects to this request as cumulative and duplicative of at least Request No. 146.

Subject to and based on these specific objections and the General Objections, Sarepta will not produce documents responsive to this request beyond those produced in response to other requests. Sarepta reserves the right to supplement this response.

**REQUEST NO. 149:**

[REDACTED]

described in Sarepta’s 10-K for the fiscal year ended December 31, 2021: “On December 21, 2019, we entered into a License, Collaboration, and Option Agreement (the “Collaboration Agreement”) with F. Hoffman-La Roche Ltd (“Roche”) pursuant to which we granted Roche an exclusive license under certain of our intellectual property rights to develop, manufacture, and commercialize SRP-9001 in all countries outside of the U.S. We retained all rights to SRP-9001 in the U.S. The transaction closed on February 4, 2020. We have entered into Amendments 1 through 8 to the Collaboration Agreement on: October 23, 2020, October 28, 2020, February 4, 2021, June 23, 2021, August 31, 2021, November 30, 2021, January 5, 2022, and January 28, 2022, respectively.”

**RESPONSE**

Sarepta incorporates its General Objections here as if set forth in full. Sarepta further objects to this request to the extent it seeks the production of documents or information protected by the attorney-client privilege, the work-product doctrine, or any other privilege or immunity, and/or claim of third-party confidentiality. Sarepta also objects to this request as overly broad and unduly burdensome to the extent it seeks “[a]ll” documents because it is not limited to documents

and information relevant to, and reasonably and proportionally directed to, the claims and defenses at issue in this case. Sarepta further objects to this request to the extent it seeks information and documents that are not relevant or reasonably and proportionally directed to the claims and defenses at issue in this case. Sarepta objects to this request to the extent it seeks documents that are publicly available, readily identifiable, and equally accessible to NS.

Subject to and based on these specific objections and the General Objections, [REDACTED]

[REDACTED] Sarepta reserves the right to supplement this response.

MORRIS, NICHOLS, ARSHT & TUNNELL LLP

OF COUNSEL:

*/s/ Megan E. Dellinger*

Charles E. Lipsey  
J. Derek McCorquindale  
FINNEGAN, HENDERSON, FARABOW,  
GARRETT & DUNNER, LLP  
1875 Explorer Street, Suite 800  
Reston, VA 20190-6023  
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GARRETT & DUNNER, LLP  
Two Seaport Lane  
Boston, MA 02210-2001  
(617) 646-1600

April 11, 2022

**CERTIFICATE OF SERVICE**

I hereby certify that on April 11, 2022, copies of the foregoing were caused to be served upon the following in the manner indicated:

Amy M. Dudash, Esquire  
MORGAN, LEWIS & BOCKIUS LLP  
1201 North Market Street, Suite 2201  
Wilmington, DE 19801  
*Attorneys for Plaintiff*

*VIA ELECTRONIC MAIL*

Amanda S. Williamson, Esquire  
Christopher J. Betti, Esquire  
Krista Vink Venegas, Esquire  
Maria E. Doukas, Esquire  
Michael T. Sikora, Esquire  
Zachary Miller, Esquire  
MORGAN, LEWIS & BOCKIUS LLP  
110 North Wacker Drive, Suite 2800  
Chicago, IL 60606  
*Attorneys for Plaintiff*

*VIA ELECTRONIC MAIL*

Jitsuro Morishita, Esquire  
MORGAN, LEWIS & BOCKIUS LLP  
16F, Marunouchi Building,  
2-4-1 Marunouchi, Chiyoda-ku  
Tokyo, 100-6316 Japan  
*Attorneys for Plaintiff*

*VIA ELECTRONIC MAIL*

*/s/ Megan E. Dellinger*

---

Megan E. Dellinger (#5739)

# Exhibit B

**Sikora, Michael T.**

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**From:** Kim, Yoonhee <Yoonhee.Kim@finnegan.com>  
**Sent:** Wednesday, April 12, 2023 4:53 PM  
**To:** Sikora, Michael T.  
**Cc:** NS District Court; Blumenfeld, Jack; Raich, William; Lee, Yoonjin; Flibbert, Michael; Chard, Beth Ann; McCorquindale, J. Derek; Kozikowski, John; Lipsey, Charles; Lipton, Alissa; Dellinger, Megan E.; Clark, Cameron; O'Quinn, Ryan  
**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

[EXTERNAL EMAIL]

Mike,

Friday 10 am ET still works for us. Please use the following dial-in:

Dial-in Number: 1 877 304 9269

Passcode: 1852625#

Mobile Quick Join: <tel://+18773049269,,1852625#>

Best regards,  
Yoonhee

**Yoonhee Kim**

Attorney at Law

Finnegan, Henderson, Farabow, Garrett & Dunner, LLP

901 New York Avenue, NW, Washington, DC 20001-4413

202.408.4214 | fax: 202.408.4400 | [yunhee.kim@finnegan.com](mailto:yunhee.kim@finnegan.com) | [www.finnegan.com](http://www.finnegan.com)

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**From:** Sikora, Michael T. <michael.sikora@morganlewis.com>  
**Sent:** Tuesday, April 11, 2023 4:41 PM  
**To:** Kim, Yoonhee <Yoonhee.Kim@finnegan.com>  
**Cc:** NS District Court <NSDistrictCourt@morganlewis.com>; Blumenfeld, Jack <JBlumenfeld@morrisnichols.com>; Raich, William <William.Raich@finnegan.com>; Lee, Yoonjin <Yoonjin.Lee@finnegan.com>; Flibbert, Michael <michael.flibbert@finnegan.com>; Chard, Beth Ann <BChard@morrisnichols.com>; McCorquindale, J. Derek <Derek.McCorquindale@finnegan.com>; Kozikowski, John <John.Kozikowski@finnegan.com>; Lipsey, Charles <charles.lipsey@finnegan.com>; Lipton, Alissa <Alissa.Lipton@finnegan.com>; Dellinger, Megan E. <mdellinger@morrisnichols.com>; Clark, Cameron <cclark@morrisnichols.com>; O'Quinn, Ryan <Ryan.O'Quinn@finnegan.com>  
**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

**EXTERNAL Email:**

Yoonhee,

We can be available on Friday at 10 ET. Please circulate a dial-in for that time if it still works for your team.

Please likewise come prepared to discuss the various issues raised in my April 4<sup>th</sup> email.

Best,

Mike

**Michael T. Sikora**

**Morgan, Lewis & Bockius LLP**

110 North Wacker Drive | Chicago, IL 60606-1511

Direct: +1.312.324.1482 | Main: +1.312.324.1000 | Cell: +1.651.233.8640 | Fax: +1.312.324.1001

michael.sikora@morganlewis.com | www.morganlewis.com

---

**From:** Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>

**Sent:** Friday, April 7, 2023 2:15 PM

**To:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>

**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>; Blumenfeld, Jack <[JBlumenfeld@morrisnichols.com](mailto:JBlumenfeld@morrisnichols.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; Chard, Beth Ann <[BChard@morrisnichols.com](mailto:BChard@morrisnichols.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Kozikowski, John <[John.Kozikowski@finnegan.com](mailto:John.Kozikowski@finnegan.com)>; Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>; Dellinger, Megan E. <[mdellinger@morrisnichols.com](mailto:mdellinger@morrisnichols.com)>; Clark, Cameron <[cclark@morrisnichols.com](mailto:cclark@morrisnichols.com)>; O'Quinn, Ryan <[Ryan.O'Quinn@finnegan.com](mailto:Ryan.O'Quinn@finnegan.com)>

**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

[EXTERNAL EMAIL]

Dear Mike:

While we disagree with the assertions and reasoning in your email, we do not feel that further written correspondence is productive at this point and look forward to articulating our views on a meet-and-confer. To that end, we are available to discuss any remaining issues next week on Tuesday, 3:30pm-5pm ET; Wednesday, 3:30pm-5pm ET; Thursday, 12pm-3pm and 4pm-5pm ET; and Friday, 9:30am-2pm and 3pm-5pm ET. Please let us know a time that works for you and your team.

To meaningfully advance the issues in the case and to minimize unnecessary intervention by the Court, please come prepared to the scheduled meet-and-confer to explain at least the following topics:

**NS/NCNP Development Documents:**

- NS's continuing failure to produce any NCNP documents in NS's possession, custody, or control for more than two months past the 1/20 production deadline.
- The multiple deficiencies in NS's document production raised in our February 16<sup>th</sup> correspondence, including the data/figures of the NS patents, missing NS documents from the 2010 to 2011 timeframe, and documents from other individuals referenced in NS synthesis documents.
- How NS's proposed search methodology will "obviate" the issues raised in our 2/16 correspondence, and a date certain by which the production will be complete.
- NS/NCNP paper documents such as laboratory notebooks in NS's possession.

**Privilege Log:**

- NS's proposal regarding privilege logs.

**Agreements with Third Parties:**

- NS's non-production of third-party agreements by the end of March. See Sikora 3/9 email ("We expect these productions to be complete by the end of March.")
- The purported relevance of the unredacted Roche agreement to any claim or defense in this case.



**Financial Records:**

- Identification by Bates number of the “detailed cost information” that you assert “has already been provided by NS.” See Sikora 4/4 email.
- The “damages issues” that NS contends for the first time will render ex-US sales data relevant in this case, particularly given NS’s prior concession that it is entitled only to “documents showing the disposition of all units of the accused products within the United States.” See Sikora 4/4 email; Sikora 2/10 email.
- Any case law supporting NS’s contention that ex-US sales data is relevant to damages in this case.
- NS’s persistent reference to accused “products” plural in this case in view of its identification of only a single accused product in its pleadings, VYONDYS 53.
- Identification by Bates number of documents or information “relating to average patient weight” for Viltepso patients. See Kim 2/21 email.

**Exondys 51 Documents:**

- The relevance of any information relating to exon 52 deletion patients beyond that produced by Sarepta at SRPT-VYDS-0213542 to SRPT-VYDS-0213543 to any claim or defense in this case.
- NS’s non-production of “documents sufficient to show its patient recruitment efforts generally” as previously promised. See Sikora 3/9 email.
- NS’s non-production of “all documents regarding a breakdown of Viltepso patients by exon deletion (e.g. exon 52 vs. other deletions),” responsive to at least Sarepta Requests for Production Nos. 40, 86, and 87. See Kim 2/21 email.

Best regards,  
Yoonhee

**Yoonhee Kim**

**Attorney at Law**

Finnegan, Henderson, Farabow, Garrett & Dunner, LLP  
901 New York Avenue, NW, Washington, DC 20001-4413  
202.408.4214 | fax: 202.408.4400 | [yoонhee.kim@finnegan.com](mailto:yoонhee.kim@finnegan.com) | [www.finnegan.com](http://www.finnegan.com)

---

**From:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>

**Sent:** Tuesday, April 4, 2023 11:54 AM

**To:** Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>

**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>; Blumenfeld, Jack <[JBlumenfeld@morrisnichols.com](mailto:JBlumenfeld@morrisnichols.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; Chard, Beth Ann <[BChard@morrisnichols.com](mailto:BChard@morrisnichols.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Kozikowski, John <[John.Kozikowski@finnegan.com](mailto:John.Kozikowski@finnegan.com)>; Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>; Dellinger, Megan E. <[mdellinger@morrisnichols.com](mailto:mdellinger@morrisnichols.com)>; Clark, Cameron <[cclark@morrisnichols.com](mailto:cclark@morrisnichols.com)>; O'Quinn, Ryan <[Ryan.O'Quinn@finnegan.com](mailto:Ryan.O'Quinn@finnegan.com)>

**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

**EXTERNAL Email:**

Dear Yoonhee,

If necessary, we are available to meet-and-confer regarding these issues on Monday, April 10 at 11 ET / 10 CT.

### **Sarepta Lab Notebooks and Development Documents**

Your email conflates sub-issues to obscure Sarepta's deficient production of, as stated below, "documents *reflecting [Sarepta's] own testing* of exon 53-directed oligonucleotides leading up to its selection of golodirsen as a candidate for clinical trials." Unless the "5 Sarepta notebooks" reflect the entirety of Sarepta's pre-clinical experiments with an oligonucleotide complementary to h53A(+36+60), we do not see how they can possibly be sufficient to "fulfill" Sarepta's discovery obligations on this issue. NS is entitled to discovery showing how Sarepta developed the accused product, Vyondys53® (golodirsen). This obligation cannot be avoided by pointing to the volume of separate UWA documents Sarepta has produced.

Additionally, your belated refusal to produce documents showing Sarepta's pre-clinical development efforts and documents regarding third-party efforts (e.g., Royal Holloway or Biomarin) in Sarepta's possession, custody and control is flatly inconsistent with at least (1) Sarepta's responses to RFP Nos. 15, 16, 17, 18, 19, 20 and 21, which agree to produce such documents; and (2) the reassurances you made during our February meet-and-confer that Sarepta would produce all PMO and 2'-O-Me-PS testing for exon 53 in its possession, custody, or control. See Sikora Email (Feb. 10, 2023). Also, as to Peter Sazani's and Ryszard Kole's work, their patent application indicates they tested SEQ ID Nos. 193, 195, and 199 from the UWA Patents. See, e.g., U.S. 2010/0130591 A1 at [0293]. Please confirm that Sarepta will promptly produce the pre-clinical development documents noted in my email below, or confirm your availability for a meet-and-confer.

### **NS Lab Notebooks and Development Documents**

We already responded to your February 16<sup>th</sup> letter on March 6, in which NS indicated it would conduct a supplemental search and production under the Default Discovery Order to obviate any of the purported deficiencies about NS and NCNP pre-clinical development documents alleged in your letter (and now repeated in your emails), and disclosed corresponding search terms for that purpose. And we also responded "soon" to the issues raised by your other then-recent correspondence, as my March 9<sup>th</sup> email below shows.

Your attempt to recharacterize our good-faith effort to quickly resolve the purported issues raised in your February 16<sup>th</sup> letter without Court intervention as some sort of tacit admission of a deficiency is not well taken. We further note that, in the three weeks preceding your Tuesday (3/27) email, Sarepta never once responded to NS's search term disclosure or otherwise indicated it would be responding. As such, we have already proceeded to apply the disclosed search terms and begun reviewing documents implicated by them, including email. We can confirm that your concern about low hit-rates from NS's choice of search terms is unfounded. We expect to begin producing documents shortly. In any event, should you believe that NS's terms are in any way deficient, the Court's ESI protocols allow Sarepta to submit additional terms. Having received none in over 30 days, NS has proceeded with its initially proposed search.

We also disagree that NS's Initial Disclosures were insufficient to provide Sarepta notice of relevant Paragraph 3(a) custodians. To the extent clarification is needed regarding non-custodial document sources, we can confirm that we will be searching the server associated with [REDACTED].

Last, NS does not currently have a certified translation for pages 1-14 corresponding to NS00066671-NS00066677 to produce nor was one ever kept in the ordinary course of business. We are happy to discuss a protocol for translations and cost sharing going forward, but NS currently has no plans to provide translations not otherwise kept in the ordinary course of business.

### **UWA Documents and Redactions**

Your email is non-responsive to the particular questions we raised. Regardless how many UWA lab notebooks Sarepta has produced, there only appears to be one laboratory notebook from any named inventor (SRPT-VYDS-0160820, for Graham McClorey). Please confirm that this notebook reflects the entirety of the experimental work conducted by the named inventors to support the UWA Patents, or that Sarepta will promptly produce any additional such laboratory notebooks by April 14.

Also, while we look forward to your response regarding more particular deficiencies and relevancy redactions, your email also does not address when Sarepta will be producing communications amongst the named inventors of the UWA Patents, communications between the named inventors of the UWA Patents and others, such as those involved in drug discovery at Sarepta and documents showing the technical information transferred from UWA to Sarepta pursuant to the November 24, 2008 Exclusive License Agreement. Again, Sarepta has already agreed to produce such documents in connection with at least RFP Nos. 5-9 and 14. Please confirm that such documents will also be produced by April 14.

We are amenable to discussing the generation and exchange of privilege logs during the meet-and-confer.

#### **Agreements with Third Parties**

While we continue to believe that Sarepta's request for NS's ex-US manufacturing agreements is overbroad and, at the very least, unnecessarily cumulative, NS can agree to conduct a reasonable search for applicable manufacturing agreements to resolve this dispute.

As Sarepta has confirmed the parties remain at an impasse regarding at least the Roche Agreement, please confirm your availability for a meet-and-confer.

#### **Regulatory and Clinical Trial Documents**

To resolve this dispute, NS can agree to produce information similar to that referenced in your email (SRPT-VYDS-0007020, SRPT-VYDS-0007051) that show the steps and reaction conditions used to chemically synthesize viltolarsen.

#### **Financial Records**

Your characterization of our correspondence requesting reasonable clarification on cost information as somehow representing an attempt "to circumvent the discovery limits" is meritless. NS has requested, and Sarepta has agreed to produce, cost information at least in response to RFP Nos. 51, 66, 68, 75, 76, and 140. Such information is indisputably relevant to damages, and detailed cost information has already been provided by NS. Please confirm Sarepta will provide the requested information.

Thank you for clarifying that the redacted information in the Weekly Sales Summary documents reflects sales to ex-US customers. We are, however, confused by Sarepta's position that such figures are not relevant to this case. We understand Vyondys53<sup>®</sup> to be manufactured in the United States, such that even ex-US sales represents income that Sarepta is receiving as a result of infringing acts in the United States (e.g., manufacture and exportation). As such, the redacted information is relevant to at least damages issues. Please confirm that (1) Sarepta will remove these redactions relating to ex-US sales; and (2) provide a date certain by which Sarepta will—to the extent it has not—produced documents sufficient to identify every unit of Vyondys53<sup>®</sup> that was made, used or sold in, imported into, or exported from the United States, even if that unit was subsequently used for activities that Sarepta does not believe would constitute infringement (e.g., ex-US sales). We understand that Sarepta has already agreed to produce such documents in response to RFP Nos. 42-49.

#### **Exondys51 Documents**

Again, we disagree with your suggestion that our correspondence seeks to "circumvent" discovery limits. The particular documents identified below fairly represent a subset of the documents responsive to, e.g., RFPs Nos. 77, 78, 79, 82, and 83 regarding the market for Duchenne Muscular Dystrophy treatments and the Accused Products' share in that market. As our correspondence shows, these particular categories were proposed to address Sarepta's concern regarding providing complete financial information regarding Exondys51.

Your email generally repeating that "Sarepta plans to produce responsive, non-privileged documents within its custody and control that relate to Sarepta's exon 51/exon 53 amenable patient population" is non-responsive to my prior email, as it does not clarify what types of information about that population Sarepta is and is not agreeing to produce. Please confirm your availability to meet-and-confer so that we may clarify the boundaries of Sarepta's proposal.

**Reproduction of SRPT\_VOL009**

As Sarepta did not agree to produce by March 24, please provide a date certain by which the reproduction will be complete.

Best,

Mike

**Michael T. Sikora**

**Morgan, Lewis & Bockius LLP**

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**From:** Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>

**Sent:** Tuesday, March 28, 2023 4:49 PM

**To:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>

**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>; Blumenfeld, Jack <[JBlumenfeld@morrisnichols.com](mailto:JBlumenfeld@morrisnichols.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; Chard, Beth Ann <[BChard@morrisnichols.com](mailto:BChard@morrisnichols.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Kozikowski, John <[John.Kozikowski@finnegan.com](mailto:John.Kozikowski@finnegan.com)>; Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>; Dellinger, Megan E. <[mdellinger@morrisnichols.com](mailto:mdellinger@morrisnichols.com)>; Clark, Cameron <[cclark@morrisnichols.com](mailto:cclark@morrisnichols.com)>; O'Quinn, Ryan <[Ryan.O'Quinn@finnegan.com](mailto:Ryan.O'Quinn@finnegan.com)>

**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

[EXTERNAL EMAIL]

**HIGHLY CONFIDENTIAL – SUBJECT TO PROTECTIVE ORDER**

Dear Mike:

We further write regarding a series of issues raised for the first time in your March 9<sup>th</sup> email regarding document productions.

**Lab Notebooks and Development Documents.** NS mischaracterizes Sarepta's document productions. To date, Sarepta has produced 61 UWA notebooks, and 5 Sarepta notebooks. Sarepta also produced a research report memorializing

██████████ "Master list" documents, corresponding to or equivalent to the requested "compound reference sheets," have also been produced to NS. In contrast, NS has produced only ██████████, and no NCNP documents despite the representations by NS and NCNP that NS already has documents from NCNP and would produce them by the substantial completion date. (See, e.g., 2023/02/16 Ltr. to NS.) NS has not produced an equivalent to the Sarepta research report or compound master list and indeed, if any party has produced "scant few" research documents, it is NS. The disparity in the volume of the parties' respective document productions speaks for itself. In any event, Sarepta's discovery obligations have been fulfilled.

With respect to NS's inquiry regarding additional "email" documents, we note that NS has produced no email documents to date. With respect to work performed by Peter Sazani and Ryszard Kole, the two researchers are not the inventors of the patents-in-suit. NS has not explained the purported relevance of their work to the issues in this case. With respect to work performed by BioMarin entities, Sarepta has no discovery obligation to produce documents from third parties. We note that Sarepta has already undertaken to produce confidential license and settlement agreements between Sarepta and BioMarin responsive to NS's request. (See, e.g., February 21<sup>st</sup> email to NS identifying Bates numbers of the BioMarin agreement production.) NS is free to obtain documents available from the USPTO interference website. To be clear, the BioMarin interference dispute itself did not involve the asserted UWA Patents in

suit and Sarepta/UWA did not submit any exon 53 skipping data. In any event, the interference documents are publicly available.

We still await your responses to our February 16<sup>th</sup> letter raising multiple deficiencies in NS's document productions to date. NS's proposed search for internal documents, which it asserted in your 3/6 email "will obviate" the issues and which we expect to respond in separate correspondence, clearly evidences the significant deficiencies in NS's own document production. Regardless, we are available to discuss NS's proposed search and in-kind production of email documents.

**UWA Documents and Redactions.** Sarepta's VOL007 production on December 1, 2022 includes [REDACTED]

. These

[REDACTED] Sarepta also produced a set of [REDACTED]

[REDACTED] . Again, in contrast to Sarepta's fulsome production—

[REDACTED]—NS has not produced lab notebooks or research reports from NCNP, including any documents from named inventors Shinichi Takeda and Tetsuya Nagata. Please confirm whether or not NS will produce these documents promptly and no later than April 4, 2023.

While we are investigating NS's questions with respect to certain notebooks and relevancy redactions, we note that NS's notebook produced at NS00066669 has no English translation to pages 1-14 corresponding to NS00066671-NS00066677. Please supplement NS's production to provide a certified English translation of those pages.

With respect to privilege redactions, the parties have not discussed or agreed to exchange a privilege log under the Protective Order. (D.I. 117, ¶ 12.4 ("The Parties shall exchange their respective privilege logs at a time to be agreed upon by the Parties following the production of documents, or as otherwise ordered by the Court.")). To avoid burdening the Court with the dispute, without prejudice to the right to redact and without waiver of privilege, we are available to discuss this issue with NS.

Best regards,  
Yoonhee

**Yoonhee Kim**

Attorney at Law

Finnegan, Henderson, Farabow, Garrett & Dunner, LLP

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**From:** Kim, Yoonhee

**Sent:** Monday, March 20, 2023 5:05 PM

**To:** 'Sikora, Michael T.' <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>

**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>; Blumenfeld, Jack <[JBlumenfeld@morrisnichols.com](mailto:JBlumenfeld@morrisnichols.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; Chard, Beth Ann <[BChard@morrisnichols.com](mailto:BChard@morrisnichols.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Kozikowski, John <[John.Kozikowski@finnegan.com](mailto:John.Kozikowski@finnegan.com)>; Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>; Dellinger, Megan E. <[mdellinger@morrisnichols.com](mailto:mdellinger@morrisnichols.com)>; Clark, Cameron <[cclark@morrisnichols.com](mailto:cclark@morrisnichols.com)>; O'Quinn, Ryan <[Ryan.O'Quinn@finnegan.com](mailto:Ryan.O'Quinn@finnegan.com)>

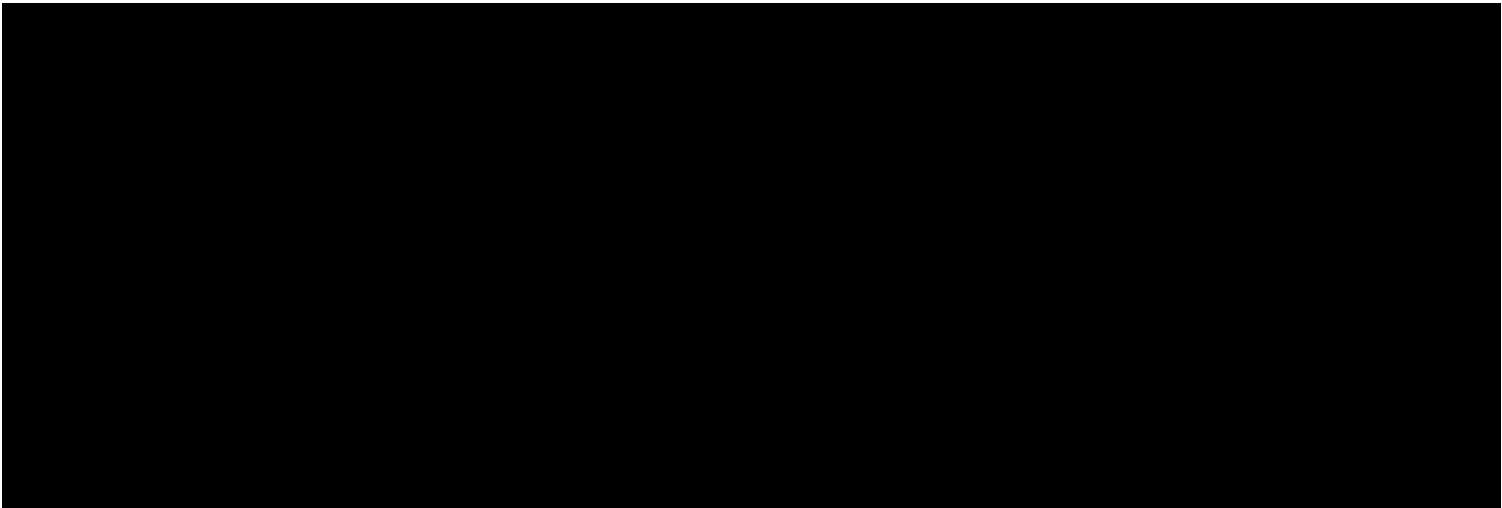
**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

**HIGHLY CONFIDENTIAL – SUBJECT TO PROTECTIVE ORDER**

Dear Mike:

We write further to our February 21<sup>st</sup> email and in response to your March 9<sup>th</sup> email below. Please provide your availability to meet and confer to discuss any remaining issues.

**Core Agreements with Third Parties:** Sarepta intends to produce the requested agreements with Orsini, Optioncare, and Bachem, subject to compliance with notice provisions, mooted this dispute. Your March 9<sup>th</sup> email requests further explanations as to why NS's ex-US manufacture of drug substance or product is relevant, without substantially addressing our February 21<sup>st</sup> email or offering any explanation as to why NS believes that it is not relevant. Any and all NS agreements related to manufacture of any viltolarsen API that enters or could potentially enter the United States are relevant to infringement and damages issues in this case, including but not limited to *Panduit* factors 3 and 4. Please confirm that NS will produce the requested agreements with previously identified entities, such as YMC Co., Ltd. (API manufacture), Ajinomoto (API manufacture), and Fuji Yakuhin Co., Ltd. (manufacture) that your email fails to acknowledge or address. If agreements with any other third parties exist relevant to distribution, manufacturing, packaging, etc., they should also be produced.



**Regulatory and Clinical Trial Documents:** Thank you for confirming that NS has produced the requested NDA documents. We confirm that Sarepta intends to produce additional FDA correspondence regarding the complete response letter, approval notices, and cover letters for amendments to the NDA sections produced, as part of an in-kind production, and already produced FDA correspondence available on the CDER website on March 6. We expect to complete production of these documents by the end of March.

With respect to NS's viltolarsen manufacturing documents, it should be apparent to NS what information may be relevant to its infringement theory. The asserted claims of the '322 patent recite specific steps and reaction conditions to make an oligomer. As previously explained in our July 26, 2022 letter, Sarepta's documents with redactions (e.g., SRPT-VYDS-0007020, SRPT-VYDS-0007051) show the steps and reaction conditions used to make golodirsén, sufficient to allow NS to evaluate the claimed limitations. Given that NS has refused to drop the '322 patent and maintains a DOE position, Sarepta is entitled to equivalent discovery to allow Sarepta to evaluate NS's viltolarsen synthesis and the manufacturing steps used in that synthesis. NS has been on notice of this request at least since November 21, 2022, and NS's continued delay is prejudicial.

**Financial Records:** NS's new request that Sarepta produce additional documents supporting and explaining each line item of a financial summary spreadsheet generated solely for the purposes of this litigation by agreement of the parties is atypical and nonsensical. Sarepta will not withhold any responsive, non-privileged documents within information previously collected that meet this criteria, but Sarepta will not generate new documents or undertake new document searches in response to this request. NS is not entitled to circumvent the discovery limits set forth in the Scheduling Order through letter correspondence.



With respect to Weekly Sales Summary documents, your request to remove redactions is directed to ex-US customers and sales information. Sarepta has the right to redact confidential commercial information not relevant to the case under the Protective Order and will not remove these redactions. Other redactions cover [REDACTED] that are not relevant to the case, and [REDACTED] information in documents [REDACTED] that the parties did not agree to produce. See 2022/12/06 Ltr. to Sikora at 3 (discussing production of [REDACTED]).

**Exondys 51 Documents:** As discussed previously, Sarepta plans to produce responsive, non-privileged documents within its custody and control that relate to Sarepta's exon 51/exon 53 amenable patient population. With respect to your four numbered subrequests—which again appear to be an attempt to circumvent court-ordered discovery limits—NS is aware of how any entity would identify exon 51/exon 53 amenable patients; they are patients with exon 52 deletions. As to the other subrequests, Sarepta plans to provide documents containing information sufficient to show at least the number of exon 51/exon 53 amenable patients to which it has provided drug, which drug was provided, the weight of the patient, and the start date and status of therapy. Sarepta plans to produce this material before the end of March.

**Sarepta, UWA and Third-Party Development Documents:** We are evaluating a series of issues raised for the first time in your March 9<sup>th</sup> email and will respond in separate correspondence.

**Reproduction of SRPT\_VOL009:** As you acknowledge, Sarepta is continuing to reproduce responsive and non-privileged documents from the inadvertently produced SRPT\_VOL009 on a rolling basis as soon as is practicable. We are continuing to investigate the issues with this production, and are releasing responsive, non-privileged documents for production as those issues are resolved. Sarepta cannot, however, commit to completing this by NS's arbitrary deadline of March 24<sup>th</sup>.

Best regards,  
Yoonhee

**Yoonhee Kim**

Attorney at Law

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**From:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>

**Sent:** Thursday, March 9, 2023 8:32 PM

**To:** Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>

**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>; Blumenfeld, Jack <[JBlumenfeld@morrisnichols.com](mailto:JBlumenfeld@morrisnichols.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; Chard, Beth Ann <[BChard@morrisnichols.com](mailto:BChard@morrisnichols.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Kozikowski, John <[John.Kozikowski@finnegan.com](mailto:John.Kozikowski@finnegan.com)>; Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>; Dellinger, Megan E. <[mdellinger@morrisnichols.com](mailto:mdellinger@morrisnichols.com)>; Clark, Cameron <[cclark@morrisnichols.com](mailto:cclark@morrisnichols.com)>

**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)


**EXTERNAL Email:**

Dear Yoonhee,

Please see below for a response to the issues your email raised, as well as a few additional issues we have identified.



**Core Agreements with Third Parties:** NS has already begun production of third-party agreements relating to U.S.-based sale and distribution of Viltepso® (specifically, with its packaging vendor, distributors, and therapy-providers), and intends to continue producing as able once it satisfies advance-notice provisions implicated by that production. We expect these productions to be complete by the end of March. Please confirm that Sarepta will likewise produce its U.S.-based agreements, including those with Orsini, Optioncare, and Bachem. As for agreements relating to ex-US manufacture of API, could you please be more specific regarding what information agreements with ex-US manufacturers contain that you contend is relevant and non-cumulative of other discovery?



**Regulatory and Clinical Trial Documents:** NS has produced the additional NDA documents, consistent with our prior correspondence, with the exception of manufacturing documents. We are considering Sarepta's position regarding manufacturing documents, but note that Sarepta has substantially redacted its own manufacturing-related documents produced to date. Could you please specify precisely what information Sarepta does and does not believe should be produced as purportedly relevant to the doctrine of equivalents, and which it deems to be properly withheld as non-responsive?

As for regulatory correspondence, we do not understand the parties' agreement at our last meet-and-confer to be limited to publicly available correspondence on the CDER website, and would not agree to modify our agreement to include that limitation. We understand NS's production to include additional non-public FDA correspondence (e.g., cover letters accompanying NDA revisions). Please confirm that Sarepta's in-kind production will contain a complete set of correspondence regarding Complete Response Letters, formal approval notices, and cover letters for amendments to the NDA sections otherwise produced, even if that correspondence is not on the CDER website.

**Financial Records:** Thank you for confirming that Sarepta will produce its ASP Reports. Please confirm that Sarepta is also producing documents sufficient to show how line items provided within its financial summary are determined (e.g., Product COGS, PAP COGs, Royalty COGs). Relatedly, we note that the Weekly Sales Summary documents produced by Sarepta contain large amounts of redacted information, even within charts that, by their titles, appear to relate solely to Vyondys53®. From the information available to us, we see no basis for withholding information regarding "Monthly Gross Sales" of Vyondys53® to some, but not other, customers. Please confirm that Sarepta will remove these redactions by March 24 or provide your availability next week to meet and confer to resolve the redaction issue. For the remaining redactions, please identify (1) what information Sarepta has redacted from this document; and (2) Sarepta's basis for contending that such information is non-responsive and/or not relevant.

**Exondys 51 Documents:** It is not clear from your response whether by "documents that would provide information sufficient to derive certain financial information relating to Exondys 51 and the number of patients cross-eligible for

Vyondys and Exondys” Sarepta agrees to produce the documents specified in my prior email, i.e., those that would allow NS to determine at least the following:

- (1) How Sarepta is identifying exon 51/exon 53 amenable patients;
- (2) How many patients/units/sales of Exondys 51 are being (a) sold; and (b) otherwise provided to exon 51/exon 53 amenable patients;
- (3) The relative amount of Exondys 51 patients/units/sales such exon 51/exon 53 amenable patients account for both (a) before launch of Vyondys53; and (b) after launch of Vyondys53; and
- (4) Any switching between Exondys 51 and Vyondys53 or Viltepso that has occurred amongst exon 51/exon 53 amenable patients.

As for your request, we can confirm that NS has conducted a reasonable search for (and will produce, to the extent not already produced) documents sufficient to show its patient recruitment efforts generally. We understand such documents would, to the extent exon 52 deletions feature in NS’s patient recruitment efforts at all, include the information you describe. Please confirm Sarepta will produce the documents noted above by March 24 or provide your availability next week to meet and confer about the basis for not providing this information.

**Sarepta, UWA and Third-Party Development Documents:** To date, Sarepta has produced scant few documents reflecting its own testing of exon 53-directed oligonucleotides leading up to its selection of golodirsen as a candidate for clinical trials. For example, Sarepta’s productions do not appear to include any email, lab notebooks, or other documents showing:

- [REDACTED]

We also have concerns regarding the UWA-related materials produced. Sarepta’s productions appear to include only [REDACTED]). Please confirm that this notebook reflects the entirety of the experimental work conducted by the named inventors to support the UWA Patents, or that Sarepta will promptly produce any additional such laboratory notebooks by March 24. Sarepta’s production also appears to lack:

- [REDACTED]

Additionally, we have not received a privilege log explaining the basis for Sarepta’s privilege redactions across its laboratory notebooks. And Sarepta’s relevancy redactions appear to be overbroad, including at least the following instances where exon 53-related data appears to have been improperly withheld:

These various development documents (and information therein) are responsive to at least RFP Nos. 5-9, 14-22. Please confirm that Sarepta will (1) produce these documents showing oligonucleotide testing leading up to the selection of golodirsen as a candidate for clinical trials; (2) produce any additional UWA laboratory notebooks; (3) produce correspondence relating to Sarepta's and UWA's work; (4) withdraw its overbroad relevancy redactions; and (5) provide a privilege log for corresponding redactions made in its laboratory notebooks by March 24 or provide your availability next week to meet and confer about the basis for not providing this information.

**Reproduction of SRPT\_VOL009:** Thank you for confirming that Sarepta has been reproducing its withdrawn production on a rolling basis. We are concerned, however, with the slow pace of Sarepta's reproduction. Over the past month and a half, it is our understanding that Sarepta has only reproduced a fraction of the original production. Please confirm this will be completed by March 24 or provide your availability next week to meet and confer about the basis for continued delays.

Best,

Mike

**Michael T. Sikora**

**Morgan, Lewis & Bockius LLP**

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**From:** Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>

**Sent:** Monday, March 6, 2023 4:36 PM

**To:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>

**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>; Blumenfeld, Jack <[JBlumenfeld@morrisnichols.com](mailto:JBlumenfeld@morrisnichols.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; Chard, Beth Ann <[BChard@morrisnichols.com](mailto:BChard@morrisnichols.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Kozikowski, John <[John.Kozikowski@finnegan.com](mailto:John.Kozikowski@finnegan.com)>; Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>; Dellinger, Megan E. <[mdellinger@morrisnichols.com](mailto:mdellinger@morrisnichols.com)>; Clark, Cameron <[cclark@morrisnichols.com](mailto:cclark@morrisnichols.com)>

**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

[EXTERNAL EMAIL]

Mike,

We have not heard from you regarding the several requests made in our February 21 email below. Please provide NS's responses at your earliest convenience, and in any event, by no later than close of business on Friday, March 10th.

Best regards,  
Yoonhee

**Yoonhee Kim**  
Attorney at Law

Finnegan, Henderson, Farabow, Garrett & Dunner, LLP  
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202.408.4214 | fax: 202.408.4400 | [yoonhee.kim@finnegan.com](mailto:yoonhee.kim@finnegan.com) | [www.finnegan.com](http://www.finnegan.com)

**From:** Kim, Yoonhee

**Sent:** Tuesday, February 21, 2023 12:11 PM

**To:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>

**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>; Blumenfeld, Jack <[JBlumenfeld@morrisnichols.com](mailto:JBlumenfeld@morrisnichols.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; Chard, Beth Ann <[BChard@morrisnichols.com](mailto:BChard@morrisnichols.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Kozikowski, John <[John.Kozikowski@finnegan.com](mailto:John.Kozikowski@finnegan.com)>; Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>; Dellinger, Megan E. <[mdellinger@morrisnichols.com](mailto:mdellinger@morrisnichols.com)>; Clark, Cameron <[cclark@morrisnichols.com](mailto:cclark@morrisnichols.com)>

**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

**HIGHLY CONFIDENTIAL – SUBJECT TO PROTECTIVE ORDER**

Dear Mike:

We write further to our February 9<sup>th</sup> email and in response to your email the next day below.

**Core Agreements with Third Parties:** In view of NS's representation that it will promptly produce "US-based agreements," Sarepta is willing to consider production of third-party agreements (and any amendments) with Orsini, Optioncare, and Bachem, if NS will produce in kind. That said, you suggested during the meet-and-confer that NS would not produce agreements for API and/or manufacturing "from overseas," specifically Japan. The location of the parties to these agreements does not necessarily determine their relevance to this case. If the agreements you referred to pertain in any way to drug substance or drug product that eventually enters the US market, they are relevant and must be produced. Please confirm that NS is not withholding any documents on these grounds, i.e., that any documents NS may be planning to withhold pertain solely to servicing ex-US markets for viltolarsen, and identify the third-party agreements NS intends to produce.

**Regulatory and Clinical Trial Documents:** We confirm that additional Sarepta regulatory and clinical trial documents were produced in SRPT\_VOL010. Out of courtesy, we identify a representative set of those documents by Bates number: SRPT-VYDS-0201593; SRPT-VYDS-0201653; SRPT-VYDS-0201702; SRPT-VYDS-0201770; SRPT-VYDS-0201819; SRPT-VYDS-0201823; SRPT-VYDS-0201883; SRPT-VYDS-0201949; SRPT-VYDS-0202016; SRPT-VYDS-0202066; SRPT-VYDS-0202135; SRPT-VYDS-0202189; SRPT-VYDS-0202235; SRPT-VYDS-0202284; SRPT-VYDS-0202395; SRPT-VYDS-0202448; SRPT-VYDS-0207246; SRPT-VYDS-0207287; SRPT-VYDS-0207420; SRPT-VYDS-0207588; SRPT-VYDS-0207841; SRPT-VYDS-0207898; SRPT-VYDS-0207932; SRPT-VYDS-0207979. Please produce without further delay NS's NDA sections or their equivalents that we requested in correspondence including our January 30, 2023 letter—NDA sections that you represented you would produce during the meet-and-confer.

With respect to FDA correspondence, upon review of NS's production, it appears that NS has only produced FDA correspondence available on the FDA CDER website. We confirm that Sarepta will likewise produce the official FDA correspondence with Sarepta available on the same website, including the formal approval letter and complete response letter, as part of an in-kind production.

**Financial Records:** Although Sarepta maintains that its prior production on January 27, 2023 of total revenue and profit numbers in the produced financial summary spreadsheet is sufficient to provide any necessary information regarding sales of Vyondys 53 in the US, Sarepta will agree to produce ASP reports provided to CMS commensurate in scope to those produced by NS. We understand that with production of these reports, the "Core Financial Records" requests from your October 31, 2022 letter are satisfied.

Regarding forecast documents, Sarepta has already produced forecasts pursuant to our prior communications. Included in those forecasts is information relating to average patient weight for Vyondys 53 patients, which is an assumption underlying the forecast data. We have not been able to identify any parallel information in NS's production. Please promptly produce this information for NS, or if you believe relevant documents have already been produced, identify them by Bates number.

**Exondys 51 Documents:** Sarepta maintains that Exondys 51 is not an accused product in this case, and financial documents and additional information regarding that product are not relevant. Nevertheless, Sarepta intends to consider production of documents that would provide information sufficient to derive certain financial information relating to Exondys 51 and the number of patients cross-eligible for Vyondys and Exondys (i.e., exon 52 deletion patients). As a condition of any such production, Sarepta would expect that NS produce 1) all documents regarding a breakdown of Viltepso patients by exon deletion (e.g. exon 52 vs. other deletions); 2) all documents regarding the recruitment of patients with exon 52 deletions to Viltepso. These documents are responsive to at least Sarepta Request for Production Nos. 40, 86, and 87 and should have already been produced. Please confirm that NS is amenable to this.

Best regards,  
Yoonhee

**Yoonhee Kim**

Attorney at Law

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**From:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>

**Sent:** Friday, February 10, 2023 5:50 PM

**To:** Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>

**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>; Blumenfeld, Jack <[JBlumenfeld@morrisnichols.com](mailto:JBlumenfeld@morrisnichols.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; Chard, Beth Ann <[BChard@morrisnichols.com](mailto:BChard@morrisnichols.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Kozikowski, John <[John.Kozikowski@finnegan.com](mailto:John.Kozikowski@finnegan.com)>; Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>; Dellinger, Megan E. <[mdellinger@morrisnichols.com](mailto:mdellinger@morrisnichols.com)>; Clark, Cameron <[cclark@morrisnichols.com](mailto:cclark@morrisnichols.com)>

**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

**EXTERNAL Email:**

Dear Yoonhee,

To clarify a few points from your email:

[REDACTED]

**Exon-Skipping Testing Data & Prosecution-Related Documents:** Yes, we reiterated our prior explanation that NS's proposed compromise for [REDACTED], and reaffirmed that NS has also produced clinical documents and data reflecting the [REDACTED] the clinical development period. And we also reiterated our continued belief that Sarepta's insistence on NS scrounging its records for instances of [REDACTED] would be disproportionate to the needs of the case. That said, we re-iterated our offer to produce certain documents relating to a discrete instance of [REDACTED] Sarepta had identified, if doing so would resolve this dispute.

You confirmed that Sarepta would be producing only P [REDACTED]

[REDACTED] We pointed out that Sarepta adopting these various carve-outs for itself demonstrates how NS's proposed compromise is reasonable and more appropriate. While Sarepta is attempting to carve out certain [REDACTED], NS's proposal is more straightforward, less burdensome, and more proportionate to the needs of the case—it focuses discovery of [REDACTED] products.

We also reiterated that Sarepta's discovery requests relating to NS's foreign patent prosecution efforts violate the parties' prior agreement that prosecution-related document production would be limited to the certified prosecution histories of the asserted patents. We explained that Sarepta's attempt to recharacterize these requests as merely seeking exon 53-related testing is belied by the fact that Sarepta's requests directly seek testing documents relating to particular patent prosecution efforts. And yes, we indicated that, should Sarepta successfully renege on its agreement regarding prosecution-related documents via a motion to compel, NS would seek reciprocal productions relating to Sarepta's patent prosecution efforts, including the Biomarin interference.

Based on Sarepta's affirmation that it seeks [REDACTED], we further indicated that NS would be willing to consider a stipulation limiting NS's ability to use the currently sought after [REDACTED], should Sarepta propose one. We invited Sarepta to reconsider its positions based on our discussion, and noted that Sarepta has not offered any proposed compromises to date. We are disappointed that Sarepta was unwilling to do so, and that your email instead declares us to be at an impasse.

**Regulatory and Clinical Trial Documents:** You did not merely state that FDA correspondence "would only be considered" if there is an in-kind production from NS. After receiving our confirmation that NS intended to make an in-kind production, you agreed that Sarepta would produce FDA correspondence, including correspondence regarding Complete Response Letters and formal approval notices. We understand that Sarepta will be making a production of such documents—please confirm it will do so.



Additionally, although you represented that investigator's brochures, DSURs, clinical trial reports, and Phase III information had been produced, we have thus far been unable to locate them in Sarepta's productions. Again, we expect this is because the documents were in SRPT\_VOL009, which we have not yet received re-production of. Please confirm either way.

**Financial Records:** Further to what you state below, we clarified that NS is entitled to documents showing the disposition of all units of the accused products within the United States, as such documents are necessary for NS to refine its infringement contentions and damages theories. You conceded that NS was entitled to information regarding units beyond those merely "sold," and acknowledged that NS has already produced the type of documents it is requesting (e.g., ASP report). We also explained that, as required regulatory-reporting documents, ASP reports should be kept in the ordinary course of business, easily accessible, and provide credible information regarding these issues. Please confirm Sarepta agrees to produce such documents promptly.

**Exondys 51 Documents:** Further to what you state below, we explained that documents providing additional contextual data would be necessary to understand the figures Sarepta proposed producing. Further to our discussion yesterday, we propose that documents be provided that would allow NS to determine at least the following:

- (1) How Sarepta is identifying exon 51/exon 53 amenable patients;
- (2) How many patients/units/sales of Exondys 51 are being (a) sold; and (b) otherwise provided to exon 51/exon 53 amenable patients;
- (3) The relative amount of Exondys 51 patients/units/sales such exon 51/exon 53 amenable patients account for both (a) before launch of Vyondys53; and (b) after launch of Vyondys53; and
- (4) Any switching between Exondys 51 and Vyondys53 or Viltepso that has occurred amongst exon 51/exon 53 amenable patients.

**NS Manufacturing Documents:** Further to what you state below, we explained that Sarepta had previously agreed that NS need not produce any manufacturing documents, and that we did not agree with the purported relevance to secondary considerations. Because Sarepta had not previously raised the purported relevant to DoE, we agreed to consider that argument, and will respond once we have been able to do so.

Best,

Mike

**Michael T. Sikora**

**Morgan, Lewis & Bockius LLP**

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Assistant: Deborah McCaskill-Walton | +1.312.324.2581 | deborah.mccaskill-walton@morganlewis.com

---

**From:** Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>

**Sent:** Thursday, February 9, 2023 3:13 PM

**To:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>

**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>; Blumenfeld, Jack <[JBlumenfeld@morrisnichols.com](mailto:JBlumenfeld@morrisnichols.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; Chard, Beth Ann <[BChard@morrisnichols.com](mailto:BChard@morrisnichols.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Kozikowski, John <[John.Kozikowski@finnegan.com](mailto:John.Kozikowski@finnegan.com)>; Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>; Dellinger, Megan E. <[mdellinger@morrisnichols.com](mailto:mdellinger@morrisnichols.com)>; Clark, Cameron <[cclark@morrisnichols.com](mailto:cclark@morrisnichols.com)>

**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

[EXTERNAL EMAIL]

**HIGHLY CONFIDENTIAL – SUBJECT TO PROTECTIVE ORDER**

Dear Mike:

We write to memorialize the meet-and-confer held yesterday, February 8, 2023. We understand that Nippon Shinyaku will also be following up regarding certain issues discussed and look forward to receiving that correspondence.

**Stipulation Regarding UWA/NCNP:** You stated that you are still considering the proposed joint stipulation we sent on December 20, 2022, and that there is a possibility of agreement. You committed to following up by next week with a call to discuss redlines to the proposed stipulation. We look forward to receiving that stipulation at your earliest convenience.

**Core Agreements with Third Parties:** You confirmed for the first time that you intend to produce U.S.-based agreements with third parties (i.e., not for API and/or manufacturing from overseas, specifically Japan), and your position is that Sarepta should do the same. We committed to following up with Sarepta on this issue given your clarified position.

**Exon-Skipping Testing Data:** You stated that your proposed [REDACTED] [REDACTED] "Your position is that the task of sifting through and collecting any [REDACTED] is disproportionate to the needs of the case. We maintained our position that any [REDACTED] [REDACTED] is highly material to the case and should be produced. We are at an impasse.

**Prosecution-Related Documents:** You took the position that you may produce any exon 53 skipping data from prosecution files only upon request from Sarepta specifying the particular testing data, which requires that Sarepta know of its existence. You requested that Sarepta reconsider our position given our view of PPMO data and asked that Sarepta propose an "acceptable middle ground." Although never having raised this issue previously, you also stated that if Sarepta wins a motion to compel on this issue, you would be cross-moving to compel production of everything, including PPMO-related data and any data from the BioMarin interference. We are at an impasse.

**Regulatory and Clinical Trial Documents:** We confirmed the January 20<sup>th</sup> document production included information from ongoing clinical trials, but stated that FDA correspondence would only be considered if there is an in-kind production from NS. You confirmed that an in-kind production of FDA correspondence should be done promptly. You also confirmed that you will produce by the end of February those NDA sections we requested in our last letter dated January 30, 2023, except for the manufacturing section (which you agreed to consider as discussed below). Please produce those documents without further delay.

**Financial Records:** You wanted us to confirm that the produced financial summary spreadsheet contains "all" units, sold and/or otherwise distributed. Regarding ASP, we explained that you have our total revenue and profits numbers already and there is no need for this type of breakdown. You countered that your request for ASP reports is to distinguish and understand where the units are going to, e.g. commercial drug vs. other drug, for infringement and damages purposes. We committed to following up with the client given your newly explained reasoning. No further agreement was made.

**Exondys 51 Documents:** Commensurate with what we proposed in our last letter, Sarepta has offered to provide sales figures for the subset of patients cross-eligible for Vyondys and Exondys (i.e., exon 52 deletion patients). You further requested "contextual data," such as what percentage of total Exondys sales that go to the dual product population. We objected to such additional data as being too easy to extrapolate other significant Exondys data. Nevertheless, both sides committed to exploring what potentially acceptable "contextualizing" data may be provided. We look forward to hearing from you on this issue.

**Manufacturing Documents:** We explained that NS's manufacturing documents are relevant to NS's doctrine of equivalents theory in addition to secondary considerations and nexus. We further explained that Sarepta is entitled to discovery of the [REDACTED]



[REDACTED]. You agreed to reconsider in light of this reasoning. We look forward to hearing from you on this issue.

Sincerely,  
Yoonhee

**Yoonhee Kim**

Attorney at Law

Finnegan, Henderson, Farabow, Garrett & Dunner, LLP  
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202.408.4214 | fax: 202.408.4400 | [yoonhee.kim@finnegan.com](mailto:yoonhee.kim@finnegan.com) | [www.finnegan.com](http://www.finnegan.com)

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**From:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>

**Sent:** Wednesday, February 8, 2023 9:06 AM

**To:** Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>; Clay, Aaron <[Aaron.Clay@finnegan.com](mailto:Aaron.Clay@finnegan.com)>

**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>; Blumenfeld, Jack <[JBlumenfeld@morrisnichols.com](mailto:JBlumenfeld@morrisnichols.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; Chard, Beth Ann <[BChard@morrisnichols.com](mailto:BChard@morrisnichols.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Kozikowski, John <[John.Kozikowski@finnegan.com](mailto:John.Kozikowski@finnegan.com)>; Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>; Dellinger, Megan E. <[mdellinger@morrisnichols.com](mailto:mdellinger@morrisnichols.com)>; Clark, Cameron <[cclark@morrisnichols.com](mailto:cclark@morrisnichols.com)>

**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

**EXTERNAL Email:**

Hi Yoonhee,

Please use the dial-in below:

Dial-In Number: +1-404-410-4502

Conference Code: 661538199

iPhone Friendly Dial-In: +1-404-410-4502, 661538199#

Best,

Mike

**Michael T. Sikora**

**Morgan, Lewis & Bockius LLP**

110 North Wacker Drive | Chicago, IL 60606-1511

Direct: +1.312.324.1482 | Main: +1.312.324.1000 | Fax: +1.312.324.1001

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Assistant: Deborah McCaskill-Walton | +1.312.324.2581 | [deborah.mccaskill-walton@morganlewis.com](mailto:deborah.mccaskill-walton@morganlewis.com)

---

**From:** Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>

**Sent:** Monday, February 6, 2023 4:41 PM

**To:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>; Clay, Aaron <[Aaron.Clay@finnegan.com](mailto:Aaron.Clay@finnegan.com)>

**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>; Blumenfeld, Jack <[JBlumenfeld@morrisnichols.com](mailto:JBlumenfeld@morrisnichols.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; Chard, Beth Ann <[BChard@morrisnichols.com](mailto:BChard@morrisnichols.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Kozikowski, John <[John.Kozikowski@finnegan.com](mailto:John.Kozikowski@finnegan.com)>; Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>; Dellinger, Megan E.

<[mdellinger@morrisnichols.com](mailto:mdellinger@morrisnichols.com)>; Clark, Cameron <[cclark@morrisnichols.com](mailto:cclark@morrisnichols.com)>

**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

[EXTERNAL EMAIL]

Mike,

We are available on Wednesday (2/8) at 10 ET. Please circulate a dial-in. Thank you.

Best regards,  
Yoonhee

**Yoonhee Kim**

Attorney at Law

Finnegan, Henderson, Farabow, Garrett & Dunner, LLP

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**From:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>

**Sent:** Monday, February 6, 2023 8:33 AM

**To:** Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>; Clay, Aaron <[Aaron.Clay@finnegan.com](mailto:Aaron.Clay@finnegan.com)>

**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>; Blumenfeld, Jack <[JBlumenfeld@morrisnichols.com](mailto:JBlumenfeld@morrisnichols.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; Chard, Beth Ann <[BChard@morrisnichols.com](mailto:BChard@morrisnichols.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Kozikowski, John <[John.Kozikowski@finnegan.com](mailto:John.Kozikowski@finnegan.com)>; Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>; Dellinger, Megan E. <[mdellinger@morrisnichols.com](mailto:mdellinger@morrisnichols.com)>

**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

**EXTERNAL Email:**

Hi Yoonhee,

We're unable to make a call today work. Are you available on Wednesday at 10 ET?

Best,

Mike

**Michael T. Sikora**

**Morgan, Lewis & Bockius LLP**

110 North Wacker Drive | Chicago, IL 60606-1511

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---

**From:** Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>

**Sent:** Wednesday, February 1, 2023 11:01 AM

**To:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>; Clay, Aaron <[Aaron.Clay@finnegan.com](mailto:Aaron.Clay@finnegan.com)>

**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>; Blumenfeld, Jack <[JBlumenfeld@morrisnichols.com](mailto:JBlumenfeld@morrisnichols.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; Chard, Beth Ann <[BChard@morrisnichols.com](mailto:BChard@morrisnichols.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Kozikowski, John <[John.Kozikowski@finnegan.com](mailto:John.Kozikowski@finnegan.com)>; Lipsey, Charles

<[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>; Dellinger, Megan E. <[mdellinger@morrisnichols.com](mailto:mdellinger@morrisnichols.com)>

**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

[EXTERNAL EMAIL]

Mike,

Thank you for providing your availability. While we are not available on Thursday, we are available to discuss the outstanding discovery issues on Monday, February 6, at 10 AM (ET). Please let us know if that time works for you and your team.

Best regards,  
Yoonhee

**Yoonhee Kim**

Attorney at Law

Finnegan, Henderson, Farabow, Garrett & Dunner, LLP

901 New York Avenue, NW, Washington, DC 20001-4413

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**From:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>

**Sent:** Tuesday, January 31, 2023 11:08 AM

**To:** Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>; Clay, Aaron <[Aaron.Clay@finnegan.com](mailto:Aaron.Clay@finnegan.com)>

**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>; Blumenfeld, Jack <[JBlumenfeld@morrisnichols.com](mailto:JBlumenfeld@morrisnichols.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; Chard, Beth Ann <[BChard@morrisnichols.com](mailto:BChard@morrisnichols.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Kozikowski, John <[John.Kozikowski@finnegan.com](mailto:John.Kozikowski@finnegan.com)>; Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>

**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

**EXTERNAL Email:**

Yoonhee,

Is your team available to meet-and-confer Thursday (2/2) at 10 ET / 9 CT?

Best,

Mike

**Michael T. Sikora**

**Morgan, Lewis & Bockius LLP**

110 North Wacker Drive | Chicago, IL 60606-1511

Direct: +1.312.324.1482 | Main: +1.312.324.1000 | Fax: +1.312.324.1001

[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com) | [www.morganlewis.com](http://www.morganlewis.com)

Assistant: Deborah McCaskill-Walton | +1.312.324.2581 | [deborah.mccaskill-walton@morganlewis.com](mailto:deborah.mccaskill-walton@morganlewis.com)

---

**From:** Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>

**Sent:** Monday, January 30, 2023 3:44 PM

**To:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>; Clay, Aaron <[Aaron.Clay@finnegan.com](mailto:Aaron.Clay@finnegan.com)>

**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>; Blumenfeld, Jack <[JBlumenfeld@morrisnichols.com](mailto:JBlumenfeld@morrisnichols.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Flibbert, Michael

<[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; Chard, Beth Ann <[BChard@morrisnichols.com](mailto:BChard@morrisnichols.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Kozikowski, John <[John.Kozikowski@finnegan.com](mailto:John.Kozikowski@finnegan.com)>; Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>

**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

[EXTERNAL EMAIL]

Mike,

Please see the attached response to your January 17, 2023, letter regarding discovery.

Regards,  
Yoonhee

**Yoonhee Kim**

Attorney at Law

Finnegan, Henderson, Farabow, Garrett & Dunner, LLP  
901 New York Avenue, NW, Washington, DC 20001-4413  
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---

**From:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>

**Sent:** Tuesday, January 17, 2023 6:31 PM

**To:** Clay, Aaron <[Aaron.Clay@finnegan.com](mailto:Aaron.Clay@finnegan.com)>

**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>; Blumenfeld, Jack <[JBlumenfeld@morrisnichols.com](mailto:JBlumenfeld@morrisnichols.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; Chard, Beth Ann <[BChard@morrisnichols.com](mailto:BChard@morrisnichols.com)>; Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Kozikowski, John <[John.Kozikowski@finnegan.com](mailto:John.Kozikowski@finnegan.com)>; Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>

**Subject:** Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

**EXTERNAL Email:**

Aaron,

Please see the attached letter.

Best,

Mike

**Michael T. Sikora**

**Morgan, Lewis & Bockius LLP**

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Direct: +1.312.324.1482 | Main: +1.312.324.1000 | Fax: +1.312.324.1001

[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com) | [www.morganlewis.com](http://www.morganlewis.com)

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exempt from disclosure under applicable law. If you believe you have received this message in error, please advise the sender by return e-mail and delete it from your mailbox. Thank you.

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**From:** [Sikora, Michael T.](#)  
**To:** [Kim, Yoonhee](#)  
**Cc:** [NS District Court](#); [Blumenfeld, Jack](#); [Raich, William](#); [Lee, Yoonjin](#); [Flibbert, Michael](#); [Chard, Beth Ann](#); [McCorquindale, J. Derek](#); [Kozikowski, John](#); [Lipsev, Charles](#); [Lipton, Alissa](#); [Dellinger, Megan E.](#); [Clark, Cameron](#); [O'Quinn, Ryan](#)  
**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)  
**Date:** Tuesday, April 4, 2023 11:54:58 AM

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**EXTERNAL Email:**

Dear Yoonhee,

If necessary, we are available to meet-and-confer regarding these issues on Monday, April 10 at 11 ET / 10 CT.

**Sarepta Lab Notebooks and Development Documents**

Your email conflates sub-issues to obscure Sarepta's deficient production of, as stated below,

"[REDACTED]  
[REDACTED]" Unless the "[REDACTED]" reflect the entirety of Sarepta's [REDACTED]  
[REDACTED] we do not see how they can possibly be sufficient to "fulfill" Sarepta's discovery obligations on this issue. NS is entitled to discovery showing how Sarepta developed the accused product, Vyondys53® (golodirsen). This obligation cannot be avoided by pointing to the volume of separate UWA documents Sarepta has produced.

Additionally, your belated refusal to produce documents showing Sarepta's pre-clinical development efforts and documents regarding third-party efforts (e.g., Royal Holloway or Biomarin) in Sarepta's possession, custody and control is flatly inconsistent with at least (1) Sarepta's responses to RFP Nos. 15, 16, 17, 18, 19, 20 and 21, which agree to produce such documents; and (2) the reassurances you made during our February meet-and-confer that Sarepta would produce all [REDACTED]  
[REDACTED] in its possession, custody, or control. See Sikora Email (Feb. 10, 2023). Also, as to Peter Sazani's and Ryszard Kole's work, their patent application indicates they tested SEQ ID Nos. 193, 195, and 199 from the UWA Patents. See, e.g., U.S. 2010/0130591 A1 at [0293]. Please confirm that Sarepta will promptly produce the pre-clinical development documents noted in my email below, or confirm your availability for a meet-and-confer.

**NS Lab Notebooks and Development Documents**

We already responded to your February 16<sup>th</sup> letter on March 6, in which NS indicated it would conduct a supplemental search and production under the Default Discovery Order to obviate any of the purported deficiencies about NS and NCNP pre-clinical development documents alleged in your letter (and now repeated in your emails), and disclosed corresponding search terms for that purpose. And we also responded "soon" to the issues raised by your other then-recent correspondence, as my March 9<sup>th</sup> email below shows.

Your attempt to recharacterize our good-faith effort to quickly resolve the purported issues raised in your February 16<sup>th</sup> letter without Court intervention as some sort of tacit admission of a deficiency is not well taken. We further note that, in the three weeks preceding your Tuesday (3/27) email,

Sarepta never once responded to NS's search term disclosure or otherwise indicated it would be responding. As such, we have already proceeded to apply the disclosed search terms and begun reviewing documents implicated by them, including email. We can confirm that your concern about low hit-rates from NS's choice of search terms is unfounded. We expect to begin producing documents shortly. In any event, should you believe that NS's terms are in any way deficient, the Court's ESI protocols allow Sarepta to submit additional terms. Having received none in over 30 days, NS has proceeded with its initially proposed search.

We also disagree that NS's Initial Disclosures were insufficient to provide Sarepta notice of relevant Paragraph 3(a) custodians. To the extent clarification is needed regarding non-custodial document sources, we can confirm that we will be searching the server associated with NS's Discovery Research Laboratories in Tsukuba, which we understand to store the pre-clinical development files for Viltepso® and the NS Patents.

Last, NS does not currently have a certified translation for pages 1-14 corresponding to NS00066671-NS00066677 to produce nor was one ever kept in the ordinary course of business. We are happy to discuss a protocol for translations and cost sharing going forward, but NS currently has no plans to provide translations not otherwise kept in the ordinary course of business.

#### **UWA Documents and Redactions**

Your email is non-responsive to the particular questions we raised. Regardless how many UWA lab notebooks Sarepta has produced, there only appears to be [REDACTED]

[REDACTED] Please confirm that [REDACTED] reflects the entirety of the experimental work conducted by the named inventors to support the UWA Patents, or that Sarepta will promptly produce any additional such laboratory notebooks by April 14.

Also, while we look forward to your response regarding more particular deficiencies and relevancy redactions, your email also does not address when Sarepta will be producing communications amongst the named inventors of the UWA Patents, communications between the named inventors of the UWA Patents and others, such as those involved in drug discovery at Sarepta and documents showing the technical information transferred from UWA to Sarepta pursuant to the November 24, 2008 Exclusive License Agreement. Again, Sarepta has already agreed to produce such documents in connection with at least RFP Nos. 5-9 and 14. Please confirm that such documents will also be produced by April 14.

We are amenable to discussing the generation and exchange of privilege logs during the meet-and-confer.

#### **Agreements with Third Parties**

While we continue to believe that Sarepta's request for NS's ex-US manufacturing agreements is overbroad and, at the very least, unnecessarily cumulative, NS can agree to conduct a reasonable search for applicable manufacturing agreements to resolve this dispute.

[REDACTED]



please confirm your availability for a meet-and-confer.

### **Regulatory and Clinical Trial Documents**

To resolve this dispute, NS can agree to produce information similar to that referenced in your email (SRPT-VYDS-0007020, SRPT-VYDS-0007051) that show the [REDACTED]

### **Financial Records**

Your characterization of our correspondence requesting reasonable clarification on cost information as somehow representing an attempt “to circumvent the discovery limits” is meritless. NS has requested, and Sarepta has agreed to produce, cost information at least in response to RFP Nos. 51, 66, 68, 75, 76, and 140. Such information is indisputably relevant to damages, and detailed cost information has already been provided by NS. Please confirm Sarepta will provide the requested information.

Thank you for clarifying that the redacted information in the Weekly Sales Summary documents reflects sales to ex-US customers. We are, however, confused by Sarepta’s position that such figures are not relevant to this case. We understand Vyondys53® to be manufactured in the United States, such that even ex-US sales represents income that Sarepta is receiving as a result of infringing acts in the United States (e.g., manufacture and exportation). As such, the redacted information is relevant to at least damages issues. Please confirm that (1) Sarepta will remove these redactions relating to ex-US sales; and (2) provide a date certain by which Sarepta will—to the extent it has not—produced documents sufficient to identify every unit of Vyondys53® that was made, used or sold in, imported into, or exported from the United States, even if that unit was subsequently used for activities that Sarepta does not believe would constitute infringement (e.g., ex-US sales). We understand that Sarepta has already agreed to produce such documents in response to RFP Nos. 42-49.

### **Exondys51 Documents**

Again, we disagree with your suggestion that our correspondence seeks to “circumvent” discovery limits. The particular documents identified below fairly represent a subset of the documents responsive to, e.g., RFPs Nos. 77, 78, 79, 82, and 83 regarding the market for Duchenne Muscular Dystrophy treatments and the Accused Products’ share in that market. As our correspondence shows, these particular categories were proposed to address Sarepta’s concern regarding providing complete financial information regarding Exondys51.

Your email generally repeating that “Sarepta plans to produce responsive, non-privileged documents within its custody and control that relate to Sarepta’s exon 51/exon 53 amenable patient population” is non-responsive to my prior email, as it does not clarify what types of information about that population Sarepta is and is not agreeing to produce. Please confirm your availability to meet-and-confer so that we may clarify the boundaries of Sarepta’s proposal.

### **Reproduction of SRPT\_VOL009**

As Sarepta did not agree to produce by March 24, please provide a date certain by which the reproduction will be complete.



Best,

Mike

**Michael T. Sikora**

**Morgan, Lewis & Bockius LLP**

110 North Wacker Drive | Chicago, IL 60606-1511

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[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com) | [www.morganlewis.com](http://www.morganlewis.com)

---

**From:** Kim, Yoonhee <Yoonhee.Kim@finnegan.com>

**Sent:** Tuesday, March 28, 2023 4:49 PM

**To:** Sikora, Michael T. <michael.sikora@morganlewis.com>

**Cc:** NS District Court <NSDistrictCourt@morganlewis.com>; Blumenfeld, Jack

<JBlumenfeld@morrisnichols.com>; Raich, William <William.Raich@finnegan.com>; Lee, Yoonjin

<Yoonjin.Lee@finnegan.com>; Flibbert, Michael <michael.flibbert@finnegan.com>; Chard, Beth Ann

<BChard@morrisnichols.com>; McCorquindale, J. Derek <Derek.McCorquindale@finnegan.com>;

Kozikowski, John <John.Kozikowski@finnegan.com>; Lipsey, Charles <charles.lipsey@finnegan.com>;

Lipton, Alissa <Alissa.Lipton@finnegan.com>; Dellinger, Megan E. <mdellinger@morrisnichols.com>;

Clark, Cameron <cclark@morrisnichols.com>; O'Quinn, Ryan <Ryan.O'Quinn@finnegan.com>

**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

[EXTERNAL EMAIL]

**HIGHLY CONFIDENTIAL – SUBJECT TO PROTECTIVE ORDER**

Dear Mike:

We further write regarding a series of issues raised for the first time in your March 9<sup>th</sup> email regarding document productions.

**Lab Notebooks and Development Documents.** NS mischaracterizes Sarepta's document productions. To date, Sarepta has produced [REDACTED] Sarepta also produced a research report memorializing [REDACTED]. "Master list" documents, corresponding to or equivalent to the requested "compound reference sheets," have also been produced to NS. In contrast, NS has produced only [REDACTED], and no NCNP documents despite the representations by NS and NCNP that NS already has documents from NCNP and would produce them by the substantial completion date. (*See, e.g.,* 2023/02/16 Ltr. to NS.) NS has not produced an equivalent to the Sarepta research report or compound master list and indeed, if any party has produced "scant few" research documents, it is NS. The disparity in the volume of the parties' respective document productions speaks for itself. In any event, Sarepta's discovery obligations have been fulfilled.

With respect to NS's inquiry regarding additional "email" documents, we note that NS has produced no email documents to date. With respect to work performed by Peter Sazani and Ryszard Kole, the two researchers are not the inventors of the patents-in-suit. NS has not explained the purported relevance of their work to the issues in this case. With respect to work performed by BioMarin

entities, Sarepta has no discovery obligation to produce documents from third parties. We note that Sarepta has already undertaken to produce confidential license and settlement agreements between Sarepta and BioMarin responsive to NS's request. (See, e.g., February 21<sup>st</sup> email to NS identifying Bates numbers of the BioMarin agreement production.) NS is free to obtain documents available from the USPTO interference website. To be clear, the BioMarin interference dispute itself did not involve the asserted UWA Patents in suit and Sarepta/UWA did not submit any exon 53 skipping data. In any event, the interference documents are publicly available.

We still await your responses to our February 16<sup>th</sup> letter raising multiple deficiencies in NS's document productions to date. NS's proposed search for internal documents, which it asserted in your 3/6 email "will obviate" the issues and which we expect to respond in separate correspondence, clearly evidences the significant deficiencies in NS's own document production. Regardless, we are available to discuss NS's proposed search and in-kind production of email documents.

**UWA Documents and Redactions.** Sarepta's VOL007 production on December 1, 2022 includes [REDACTED]. These [REDACTED]. Sarepta also produced [REDACTED]. Again, in contrast to Sarepta's fulsome production—which includes [REDACTED]—NS has not produced lab notebooks or research reports from NCNP, including any documents from named inventors Shinichi Takeda and Tetsuya Nagata. Please confirm whether or not NS will produce these documents promptly and no later than April 4, 2023.

While we are investigating NS's questions with respect to certain notebooks and relevancy redactions, we note that NS's notebook produced at NS00066669 has no English translation to pages 1-14 corresponding to NS00066671-NS00066677. Please supplement NS's production to provide a certified English translation of those pages.

With respect to privilege redactions, the parties have not discussed or agreed to exchange a privilege log under the Protective Order. (D.I. 117, ¶ 12.4 ("The Parties shall exchange their respective privilege logs at a time to be agreed upon by the Parties following the production of documents, or as otherwise ordered by the Court.")). To avoid burdening the Court with the dispute, without prejudice to the right to redact and without waiver of privilege, we are available to discuss this issue with NS.

Best regards,  
Yoonhee

**Yoonhee Kim**  
Attorney at Law

Finnegan, Henderson, Farabow, Garrett & Dunner, LLP  
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202.408.4214 | fax: 202.408.4400 | [yoonyhee.kim@finnegan.com](mailto:yoonyhee.kim@finnegan.com) | [www.finnegan.com](http://www.finnegan.com)

---

**From:** Kim, Yoonhee

**Sent:** Monday, March 20, 2023 5:05 PM

**To:** 'Sikora, Michael T.' <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>

**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>; Blumenfeld, Jack <[JBlumenfeld@morrisnichols.com](mailto:JBlumenfeld@morrisnichols.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; Chard, Beth Ann <[BChard@morrisnichols.com](mailto:BChard@morrisnichols.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Kozikowski, John <[John.Kozikowski@finnegan.com](mailto:John.Kozikowski@finnegan.com)>; Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>; Dellinger, Megan E. <[mdellinger@morrisnichols.com](mailto:mdellinger@morrisnichols.com)>; Clark, Cameron <[cclark@morrisnichols.com](mailto:cclark@morrisnichols.com)>; O'Quinn, Ryan <[Ryan.O'Quinn@finnegan.com](mailto:Ryan.O'Quinn@finnegan.com)>

**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

**HIGHLY CONFIDENTIAL – SUBJECT TO PROTECTIVE ORDER**

Dear Mike:

We write further to our February 21<sup>st</sup> email and in response to your March 9<sup>th</sup> email below. Please provide your availability to meet and confer to discuss any remaining issues.

**Core Agreements with Third Parties:** Sarepta intends to produce the requested agreements with Orsini, Optioncare, and Bachem, subject to compliance with notice provisions, mooted this dispute. Your March 9<sup>th</sup> email requests further explanations as to why NS's ex-US manufacture of drug substance or product is relevant, without substantially addressing our February 21<sup>st</sup> email or offering any explanation as to why NS believes that it is not relevant. Any and all NS agreements related to manufacture of any viltolarsen API that enters or could potentially enter the United States are relevant to infringement and damages issues in this case, including but not limited to *Panduit* factors 3 and 4. Please confirm that NS will produce the requested agreements with previously identified entities, such as [REDACTED] manufacture), [REDACTED] manufacture), and [REDACTED] (manufacture) that your email fails to acknowledge or address. If agreements with any other third parties exist relevant to distribution, manufacturing, packaging, etc., they should also be produced.

Sarepta reiterates that NS's request for the full Roche agreement or documents relating to "treatments for DMD beyond exon 53-directed oligonucleotides" is not relevant or proportional to the needs of the case. The parties have negotiated the scope of document production relating to Exondys (as discussed separately below), while continuing to disagree regarding the relevance of the Roche agreement. Sarepta produced a redacted version of the Roche agreement responsive to NS's discovery request and identified the Bates number of that document for your consideration as a courtesy. NS's understanding that the Roche agreement is relevant because it [REDACTED] [REDACTED] is incorrect. It should be clear upon review of that document that Roche was granted [REDACTED] [REDACTED]. See SRPT-VYDS-0204720 at 29 [REDACTED] [REDACTED]; 31 (" [REDACTED] The

Roche agreement also involves other products currently under development by Sarepta, that are not the subject of this litigation and therefore are not relevant or proportional to the needs of the case. Sarepta maintains the position articulated in correspondence including its February 21<sup>st</sup> email that the unredacted Roche agreement will not be produced.

**Regulatory and Clinical Trial Documents:** Thank you for confirming that NS has produced the requested NDA documents. We confirm that Sarepta intends to produce additional FDA correspondence regarding the complete response letter, approval notices, and cover letters for amendments to the NDA sections produced, as part of an in-kind production, and already produced FDA correspondence available on the CDER website on March 6. We expect to complete production of these documents by the end of March.

With respect to NS's viltolarsen manufacturing documents, it should be apparent to NS what information may be relevant to its infringement theory. The asserted claims of the '322 patent recite specific steps and reaction conditions to make an oligomer. As previously explained in our July 26, 2022 letter, Sarepta's documents with redactions (e.g., SRPT-VYDS-0007020, SRPT-VYDS-0007051) show the steps and reaction conditions used to make golodirsen, sufficient to allow NS to evaluate the claimed limitations. Given that NS has refused to drop the '322 patent and maintains a DOE position, Sarepta is entitled to equivalent discovery to allow Sarepta to evaluate NS's viltolarsen synthesis and the manufacturing steps used in that synthesis. NS has been on notice of this request at least since November 21, 2022, and NS's continued delay is prejudicial.

**Financial Records:** NS's new request that Sarepta produce additional documents supporting and explaining each line item of a financial summary spreadsheet generated solely for the purposes of this litigation by agreement of the parties is atypical and nonsensical. Sarepta will not withhold any responsive, non-privileged documents within information previously collected that meet this criteria, but Sarepta will not generate new documents or undertake new document searches in response to this request. NS is not entitled to circumvent the discovery limits set forth in the Scheduling Order through letter correspondence.

With respect to Weekly Sales Summary documents, your request to remove redactions is directed to ex-US customers and sales information. Sarepta has the right to redact confidential commercial information not relevant to the case under the Protective Order and will not remove these redactions. Other redactions cover weekly sales and consolidated summaries related to Exondys and Amondys products that are not relevant to the case, and forecast-related information in documents dated later than August 2020 that the parties did not agree to produce. See 2022/12/06 Ltr. to Sikora at 3 (discussing production of forecast documents from before the launch of each of the accused products).

**Exondys 51 Documents:** As discussed previously, Sarepta plans to produce responsive, non-privileged documents within its custody and control that relate to Sarepta's exon 51/exon 53 amenable patient population. With respect to your four numbered subrequests—which again appear to be an attempt to circumvent court-ordered discovery limits—NS is aware of how any entity would identify exon 51/exon 53 amenable patients; they are patients with exon 52 deletions. As to the other subrequests, Sarepta plans to provide documents containing information sufficient

to show at least the number of exon 51/exon 53 amenable patients to which it has provided drug, which drug was provided, the weight of the patient, and the start date and status of therapy. Sarepta plans to produce this material before the end of March.

**Sarepta, UWA and Third-Party Development Documents:** We are evaluating a series of issues raised for the first time in your March 9<sup>th</sup> email and will respond in separate correspondence.

**Reproduction of SRPT\_VOL009:** As you acknowledge, Sarepta is continuing to reproduce responsive and non-privileged documents from the inadvertently produced SRPT\_VOL009 on a rolling basis as soon as is practicable. We are continuing to investigate the issues with this production, and are releasing responsive, non-privileged documents for production as those issues are resolved. Sarepta cannot, however, commit to completing this by NS's arbitrary deadline of March 24<sup>th</sup>.

Best regards,  
Yoonhee

**Yoonhee Kim**

Attorney at Law

Finnegan, Henderson, Farabow, Garrett & Dunner, LLP

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**From:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>

**Sent:** Thursday, March 9, 2023 8:32 PM

**To:** Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>

**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>; Blumenfeld, Jack <[JBlumenfeld@morrisnichols.com](mailto:JBlumenfeld@morrisnichols.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; Chard, Beth Ann <[BChard@morrisnichols.com](mailto:BChard@morrisnichols.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Kozikowski, John <[John.Kozikowski@finnegan.com](mailto:John.Kozikowski@finnegan.com)>; Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>; Dellinger, Megan E. <[mdellinger@morrisnichols.com](mailto:mdellinger@morrisnichols.com)>; Clark, Cameron <[cclark@morrisnichols.com](mailto:cclark@morrisnichols.com)>

**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

**EXTERNAL Email:**

Dear Yoonhee,

Please see below for a response to the issues your email raised, as well as a few additional issues we have identified.

**Core Agreements with Third Parties:** NS has already begun production of third-party agreements relating to U.S.-based sale and distribution of Viltepso® (specifically, with its packaging vendor, distributors, and therapy-providers), and intends to continue producing as able once it satisfies advance-notice provisions implicated by that production. We expect these productions to be

complete by the end of March. Please confirm that Sarepta will likewise produce its U.S.-based agreements, including those with Orsini, Optioncare, and Bachem. As for agreements relating to ex-US manufacture of API, could you please be more specific regarding what information agreements with ex-US manufacturers contain that you contend is relevant and non-cumulative of other discovery?

Thank you for identifying certain agreements. However, it appears we are at a fundamental impasse regarding whether documents “relating to treatments for DMD beyond exon 53-directed oligonucleotides” are relevant to this case. From our review of documents Sarepta has provided to date, it appears that Sarepta has inappropriately withheld responsive and relevant information regarding current and future DMD products that affect the market for both accused products in this case. As we have discussed, Exondys51® has an overlapping patient population, and Sarepta’s microdystrophin gene-therapy product (SRP-9001) being developed—for which we understand Sarepta is pursuing an indication encompassing all ambulant individuals with DMD—plainly does as well. As an example, documents produced in SRPT\_VOL013 appear to provide [REDACTED], yet have extensive redactions that appear to withhold any information relating to these competitive products. We do not understand Sarepta’s position regarding the Roche agreement, which you concede [REDACTED]. Your characterization that this agreement “primarily involves other products” also demonstrates the problem with Sarepta’s refusal to provide the full agreement. NS is entitled to review the agreement and make that judgment for itself, particularly given that the “other products” to which you refer seem to include the Exondys51® and SRP-9001 products that are relevant to the market for exon 53-amenable patients. Given the parties’ prior correspondence on this issue, we expect that we are at an impasse. Please confirm whether Sarepta will reconsider its position and, if not, please provide your availability next week so that we may seek the Court’s intervention to resolve this issue.

**Regulatory and Clinical Trial Documents:** NS has produced the additional NDA documents, consistent with our prior correspondence, with the exception of manufacturing documents. We are considering Sarepta’s position regarding manufacturing documents, but note that Sarepta has substantially redacted its own manufacturing-related documents produced to date. Could you please specify precisely what information Sarepta does and does not believe should be produced as purportedly relevant to the doctrine of equivalents, and which it deems to be properly withheld as non-responsive?

As for regulatory correspondence, we do not understand the parties’ agreement at our last meet-and-confer to be limited to publicly available correspondence on the CDER website, and would not agree to modify our agreement to include that limitation. We understand NS’s production to include additional non-public FDA correspondence (e.g., cover letters accompanying NDA revisions). Please confirm that Sarepta’s in-kind production will contain a complete set of correspondence regarding Complete Response Letters, formal approval notices, and cover letters for amendments to the NDA sections otherwise produced, even if that correspondence is not on the CDER website.

**Financial Records:** Thank you for confirming that Sarepta will produce its ASP Reports. Please confirm that Sarepta is also producing documents sufficient to show how line items provided within

its financial summary are determined (e.g., Product COGS, PAP COGs, Royalty COGs). Relatedly, we note that the Weekly Sales Summary documents produced by Sarepta contain large amounts of redacted information, even within charts that, by their titles, appear to relate solely to Vyondys53®. From the information available to us, we see no basis for withholding information regarding “Monthly Gross Sales” of Vyondys53® to some, but not other, customers. Please confirm that Sarepta will remove these redactions by March 24 or provide your availability next week to meet and confer to resolve the redaction issue. For the remaining redactions, please identify (1) what information Sarepta has redacted from this document; and (2) Sarepta’s basis for contending that such information is non-responsive and/or not relevant.

**Exondys 51 Documents:** It is not clear from your response whether by “documents that would provide information sufficient to derive certain financial information relating to Exondys 51 and the number of patients cross-eligible for Vyondys and Exondys” Sarepta agrees to produce the documents specified in my prior email, i.e., those that would allow NS to determine at least the following:

1. How Sarepta is identifying exon 51/exon 53 amenable patients;
2. How many patients/units/sales of Exondys 51 are being (a) sold; and (b) otherwise provided to exon 51/exon 53 amenable patients;
3. The relative amount of Exondys 51 patients/units/sales such exon 51/exon 53 amenable patients account for both (a) before launch of Vyondys53; and (b) after launch of Vyondys53; and
4. Any switching between Exondys 51 and Vyondys53 or Viltepso that has occurred amongst exon 51/exon 53 amenable patients.

As for your request, we can confirm that NS has conducted a reasonable search for (and will produce, to the extent not already produced) documents sufficient to show its patient recruitment efforts generally. We understand such documents would, to the extent exon 52 deletions feature in NS’s patient recruitment efforts at all, include the information you describe. Please confirm Sarepta will produce the documents noted above by March 24 or provide your availability next week to meet and confer about the basis for not providing this information.

**Sarepta, UWA and Third-Party Development Documents:** To date, Sarepta has produced scant few documents reflecting its own testing of exon 53-directed oligonucleotides leading up to its selection of golodirsen as a candidate for clinical trials. For example, Sarepta’s productions do not appear to include any email, lab notebooks, or other documents showing:

- [REDACTED]
- [REDACTED]
- [REDACTED]
- [REDACTED]
- [REDACTED]
- [REDACTED]
- [REDACTED]
- [REDACTED]



- [REDACTED]

We also have concerns regarding the UWA-related materials produced. Sarepta's productions appear to include only one laboratory notebook from any named inventor (SRPT-VYDS-0160820, for Graham McClorey). Please confirm that this notebook reflects the entirety of the experimental work conducted by the named inventors to support the UWA Patents, or that Sarepta will promptly produce any additional such laboratory notebooks by March 24. Sarepta's production also appears to lack:

- [REDACTED]
- [REDACTED]
- [REDACTED]
- [REDACTED]
- [REDACTED]
- [REDACTED]
- [REDACTED]

Additionally, we have not received a privilege log explaining the basis for Sarepta's privilege redactions across its laboratory notebooks. And Sarepta's relevancy redactions appear to be overbroad, including at least the following instances where exon 53-related data appears to have been improperly withheld:

- [REDACTED]
- [REDACTED]
- [REDACTED]

These various development documents (and information therein) are responsive to at least RFP Nos. 5-9, 14-22. Please confirm that Sarepta will (1) produce these documents showing oligonucleotide testing leading up to the selection of golodirsen as a candidate for clinical trials; (2) produce any additional UWA laboratory notebooks; (3) produce correspondence relating to Sarepta's and UWA's work; (4) withdraw its overbroad relevancy redactions; and (5) provide a privilege log for corresponding redactions made in its laboratory notebooks by March 24 or provide your availability next week to meet and confer about the basis for not providing this information.

**Reproduction of SRPT\_VOL009:** Thank you for confirming that Sarepta has been reproducing its withdrawn production on a rolling basis. We are concerned, however, with the slow pace of Sarepta's reproduction. Over the past month and a half, it is our understanding that Sarepta has only reproduced a fraction of the original production. Please confirm this will be completed by March 24 or provide your availability next week to meet and confer about the basis for continued delays.



Best,

Mike

**Michael T. Sikora**

**Morgan, Lewis & Bockius LLP**

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**From:** Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>

**Sent:** Monday, March 6, 2023 4:36 PM

**To:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>

**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>; Blumenfeld, Jack <[JBlumenfeld@morrisnichols.com](mailto:JBlumenfeld@morrisnichols.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; Chard, Beth Ann <[BChard@morrisnichols.com](mailto:BChard@morrisnichols.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Kozikowski, John <[John.Kozikowski@finnegan.com](mailto:John.Kozikowski@finnegan.com)>; Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>; Dellinger, Megan E. <[mdellinger@morrisnichols.com](mailto:mdellinger@morrisnichols.com)>; Clark, Cameron <[cclark@morrisnichols.com](mailto:cclark@morrisnichols.com)>

**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

[EXTERNAL EMAIL]

Mike,

We have not heard from you regarding the several requests made in our February 21 email below. Please provide NS's responses at your earliest convenience, and in any event, by no later than close of business on Friday, March 10th.

Best regards,  
Yoonhee

**Yoonhee Kim**

**Attorney at Law**

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**From:** Kim, Yoonhee

**Sent:** Tuesday, February 21, 2023 12:11 PM

**To:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>

**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>; Blumenfeld, Jack <[JBlumenfeld@morrisnichols.com](mailto:JBlumenfeld@morrisnichols.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; Chard, Beth Ann <[BChard@morrisnichols.com](mailto:BChard@morrisnichols.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>;

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**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

**HIGHLY CONFIDENTIAL – SUBJECT TO PROTECTIVE ORDER**

Dear Mike:

We write further to our February 9<sup>th</sup> email and in response to your email the next day below.

**Core Agreements with Third Parties:** In view of NS's representation that it will promptly produce "US-based agreements," Sarepta is willing to consider production of third-party agreements (and any amendments) with Orsini, Optioncare, and Bachem, if NS will produce in kind. That said, you suggested during the meet-and-confer that NS would not produce agreements for API and/or manufacturing "from overseas," specifically Japan. The location of the parties to these agreements does not necessarily determine their relevance to this case. If the agreements you referred to pertain in any way to drug substance or drug product that eventually enters the US market, they are relevant and must be produced. Please confirm that NS is not withholding any documents on these grounds, i.e., that any documents NS may be planning to withhold pertain solely to servicing ex-US markets for viltolarsen, and identify the third-party agreements NS intends to produce.

The BioMarin, Royal Holloway, and redacted Roche agreements were produced in SRPT\_VOL010 on January 20, 2023. As a courtesy, we identify these agreements by Bates number: SRPT-VYDS-0206683; SRPT-VYDS-0207088; SRPT-VYDS-0207092; SRPT-VYDS-0207178; SRPT-VYDS-0204720. To clarify, Sarepta withdrew inadvertently produced VOL009, and has been reproducing portions of VOL009, with appropriate redactions, on a rolling basis (see, e.g., VOL012). Absent further explanation, Sarepta does not agree to produce a non-redacted version of the Roche agreement, as this pertains to the Ex-US market, primarily involves other products, and does not convey a license to commercialize Vyondys. Further, Sarepta maintains that "licenses relating to treatments for DMD beyond exon 53-directed oligonucleotides" are not relevant to this case, and will not be produced.

**Regulatory and Clinical Trial Documents:** We confirm that additional Sarepta regulatory and clinical trial documents were produced in SRPT\_VOL010. Out of courtesy, we identify a representative set of those documents by Bates number: SRPT-VYDS-0201593; SRPT-VYDS-0201653; SRPT-VYDS-0201702; SRPT-VYDS-0201770; SRPT-VYDS-0201819; SRPT-VYDS-0201823; SRPT-VYDS-0201883; SRPT-VYDS-0201949; SRPT-VYDS-0202016; SRPT-VYDS-0202066; SRPT-VYDS-0202135; SRPT-VYDS-0202189; SRPT-VYDS-0202235; SRPT-VYDS-0202284; SRPT-VYDS-0202395; SRPT-VYDS-0202448; SRPT-VYDS-0207246; SRPT-VYDS-0207287; SRPT-VYDS-0207420; SRPT-VYDS-0207588; SRPT-VYDS-0207841; SRPT-VYDS-0207898; SRPT-VYDS-0207932; SRPT-VYDS-0207979. Please produce without further delay NS's NDA sections or their equivalents that we requested in correspondence including our January 30, 2023 letter—NDA sections that you represented you would produce during the meet-and-confer.

With respect to FDA correspondence, upon review of NS's production, it appears that NS has only

produced FDA correspondence available on the FDA CDER website. We confirm that Sarepta will likewise produce the official FDA correspondence with Sarepta available on the same website, including the formal approval letter and complete response letter, as part of an in-kind production.

**Financial Records:** Although Sarepta maintains that its prior production on January 27, 2023 of total revenue and profit numbers in the produced financial summary spreadsheet is sufficient to provide any necessary information regarding sales of Vyondys 53 in the US, Sarepta will agree to produce ASP reports provided to CMS commensurate in scope to those produced by NS. We understand that with production of these reports, the “Core Financial Records” requests from your October 31, 2022 letter are satisfied.

Regarding forecast documents, Sarepta has already produced forecasts pursuant to our prior communications. Included in those forecasts is information relating to average patient weight for Vyondys 53 patients, which is an assumption underlying the forecast data. We have not been able to identify any parallel information in NS’s production. Please promptly produce this information for NS, or if you believe relevant documents have already been produced, identify them by Bates number.

**Exondys 51 Documents:** Sarepta maintains that Exondys 51 is not an accused product in this case, and financial documents and additional information regarding that product are not relevant. Nevertheless, Sarepta intends to consider production of documents that would provide information sufficient to derive certain financial information relating to Exondys 51 and the number of patients cross-eligible for Vyondys and Exondys (i.e., exon 52 deletion patients). As a condition of any such production, Sarepta would expect that NS produce 1) all documents regarding a breakdown of Viltepso patients by exon deletion (e.g. exon 52 vs. other deletions); 2) all documents regarding the recruitment of patients with exon 52 deletions to Viltepso. These documents are responsive to at least Sarepta Request for Production Nos. 40, 86, and 87 and should have already been produced. Please confirm that NS is amenable to this.

Best regards,  
Yoonhee

**Yoonhee Kim**

Attorney at Law

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**From:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>

**Sent:** Friday, February 10, 2023 5:50 PM

**To:** Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>

**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>; Blumenfeld, Jack <[JBlumenfeld@morrisnichols.com](mailto:JBlumenfeld@morrisnichols.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; Chard, Beth Ann <[BChard@morrisnichols.com](mailto:BChard@morrisnichols.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>;

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**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

**EXTERNAL Email:**

Dear Yoonhee,

To clarify a few points from your email:

**Core Agreements with Third Parties:** We disagree that our confirmation represented the “first time” we indicated our intent for these documents to be produced by both parties. Nevertheless, we look forward to prompt confirmation from Sarepta that it will produce the requested agreements, including those with Orsini, Optioncare, Bachem, and Roche. To the extent that any such agreements (apart from those with UWA) have already been produced, please identify them. Although you represented that agreements with Biomarin and Royal Holloway have been produced, along with a redacted version of the Roche agreement, we have thus far been unable to locate them in Sarepta’s production. We expect this is because the agreements were in SRPT\_VOL009, which we have not yet received re-production of. Please confirm either way. Additionally, please let us know whether Sarepta will produce an unredacted version of the Roche agreement, as well as other licenses relating to treatments for DMD beyond exon 53-directed oligonucleotides.

**Exon-Skipping Testing Data & Prosecution-Related Documents:** Yes, we reiterated our prior explanation that NS’s proposed compromise for *in vitro* exon-skipping data encompasses the parties’ core pre-clinical development period for their exon-53 PMO products, and reaffirmed that NS has also produced clinical documents and data reflecting the subsequent development work on Viltepso occurring after it moved into the clinical development period. And we also reiterated our continued belief that Sarepta’s insistence on NS scrounging its records for instances of *in vitro* testing performed after development efforts shifted to *in vivo* clinical trials would be disproportionate to the needs of the case. That said, we re-iterated our offer to produce certain documents relating to a discrete instance of subsequent *in vitro* testing Sarepta had identified, if doing so would resolve this dispute.

You confirmed that Sarepta would be producing only [REDACTED]

[REDACTED] We pointed out that Sarepta adopting these various carve-outs for itself demonstrates how NS’s proposed compromise is reasonable and more appropriate. While Sarepta is attempting to carve out certain [REDACTED] on an ad-hoc basis, NS’s proposal is more straightforward, less burdensome, and more proportionate to the needs of the case—it focuses discovery of [REDACTED]

██████████

We also reiterated that Sarepta's discovery requests relating to NS's foreign patent prosecution efforts violate the parties' prior agreement that prosecution-related document production would be limited to the certified prosecution histories of the asserted patents. We explained that Sarepta's attempt to recharacterize these requests as merely seeking exon 53-related testing is belied by the fact that Sarepta's requests directly seek testing documents relating to particular patent prosecution efforts. And yes, we indicated that, should Sarepta successfully renege on its agreement regarding prosecution-related documents via a motion to compel, NS would seek reciprocal productions relating to Sarepta's patent prosecution efforts, including the Biomarin interference.

Based on Sarepta's affirmation that it seeks *in vitro* data for the secondary consideration of unexpected results, we further indicated that NS would be willing to consider a stipulation limiting NS's ability to use the currently sought after *in vitro* data for the purpose of establishing unexpected results, should Sarepta propose one. We invited Sarepta to reconsider its positions based on our discussion, and noted that Sarepta has not offered any proposed compromises to date. We are disappointed that Sarepta was unwilling to do so, and that your email instead declares us to be at an impasse.

**Regulatory and Clinical Trial Documents:** You did not merely state that FDA correspondence "would only be considered" if there is an in-kind production from NS. After receiving our confirmation that NS intended to make an in-kind production, you agreed that Sarepta would produce FDA correspondence, including correspondence regarding Complete Response Letters and formal approval notices. We understand that Sarepta will be making a production of such documents—please confirm it will do so.

Additionally, although you represented that investigator's brochures, DSURs, clinical trial reports, and Phase III information had been produced, we have thus far been unable to locate them in Sarepta's productions. Again, we expect this is because the documents were in SRPT\_VOL009, which we have not yet received re-production of. Please confirm either way.

**Financial Records:** Further to what you state below, we clarified that NS is entitled to documents showing the disposition of all units of the accused products within the United States, as such documents are necessary for NS to refine its infringement contentions and damages theories. You conceded that NS was entitled to information regarding units beyond those merely "sold," and acknowledged that NS has already produced the type of documents it is requesting (e.g., ASP report). We also explained that, as required regulatory-reporting documents, ASP reports should be kept in the ordinary course of business, easily accessible, and provide credible information regarding these issues. Please confirm Sarepta agrees to produce such documents promptly.

**Exondys 51 Documents:** Further to what you state below, we explained that documents providing additional contextual data would be necessary to understand the figures Sarepta proposed producing. Further to our discussion yesterday, we propose that documents be provided that would allow NS to determine at least the following:

1. How Sarepta is identifying exon 51/exon 53 amenable patients;

2. How many patients/units/sales of Exondys 51 are being (a) sold; and (b) otherwise provided to exon 51/exon 53 amenable patients;
3. The relative amount of Exondys 51 patients/units/sales such exon 51/exon 53 amenable patients account for both (a) before launch of Vyondys53; and (b) after launch of Vyondys53; and
4. Any switching between Exondys 51 and Vyondys53 or Viltepso that has occurred amongst exon 51/exon 53 amenable patients.

**NS Manufacturing Documents:** Further to what you state below, we explained that Sarepta had previously agreed that NS need not produce any manufacturing documents, and that we did not agree with the purported relevance to secondary considerations. Because Sarepta had not previously raised the purported relevant to DoE, we agreed to consider that argument, and will respond once we have been able to do so.

Best,

Mike

**Michael T. Sikora**

**Morgan, Lewis & Bockius LLP**

110 North Wacker Drive | Chicago, IL 60606-1511

Direct: +1.312.324.1482 | Main: +1.312.324.1000 | Fax: +1.312.324.1001

[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com) | [www.morganlewis.com](http://www.morganlewis.com)

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---

**From:** Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>

**Sent:** Thursday, February 9, 2023 3:13 PM

**To:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>

**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>; Blumenfeld, Jack <[JBlumenfeld@morrisnichols.com](mailto:JBlumenfeld@morrisnichols.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; Chard, Beth Ann <[BChard@morrisnichols.com](mailto:BChard@morrisnichols.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Kozikowski, John <[John.Kozikowski@finnegan.com](mailto:John.Kozikowski@finnegan.com)>; Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>; Dellinger, Megan E. <[mdellinger@morrisnichols.com](mailto:mdellinger@morrisnichols.com)>; Clark, Cameron <[cclark@morrisnichols.com](mailto:cclark@morrisnichols.com)>

**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

[EXTERNAL EMAIL]

**HIGHLY CONFIDENTIAL – SUBJECT TO PROTECTIVE ORDER**

Dear Mike:

We write to memorialize the meet-and-confer held yesterday, February 8, 2023. We understand that Nippon Shinyaku will also be following up regarding certain issues discussed and look forward to receiving that correspondence.

**Stipulation Regarding UWA/NCNP:** You stated that you are still considering the proposed joint

stipulation we sent on December 20, 2022, and that there is a possibility of agreement. You committed to following up by next week with a call to discuss redlines to the proposed stipulation. We look forward to receiving that stipulation at your earliest convenience.

**Core Agreements with Third Parties:** You confirmed for the first time that you intend to produce U.S.-based agreements with third parties (i.e., not for API and/or manufacturing from overseas, specifically Japan), and your position is that Sarepta should do the same. We committed to following up with Sarepta on this issue given your clarified position.

**Exon-Skipping Testing Data:** You stated that your proposed cut-off of exon 53 skipping data based on the date that clinical trials began is to encompass the “core development period.” Your position is that the task of sifting through and collecting any pre-clinical testing done after clinical work began is disproportionate to the needs of the case. We maintained our position that any exon 53 skipping data generated before the filing date of the complaint involving PMOs or 2'-O-Me-PS is highly material to the case and should be produced. We are at an impasse.

**Prosecution-Related Documents:** You took the position that you may produce any exon 53 skipping data from prosecution files only upon request from Sarepta specifying the particular testing data, which requires that Sarepta know of its existence. You requested that Sarepta reconsider our position given our view of PPMO data and asked that Sarepta propose an “acceptable middle ground.” Although never having raised this issue previously, you also stated that if Sarepta wins a motion to compel on this issue, you would be cross-moving to compel production of everything, including PPMO-related data and any data from the BioMarin interference. We are at an impasse.

**Regulatory and Clinical Trial Documents:** We confirmed the January 20<sup>th</sup> document production included information from ongoing clinical trials, but stated that FDA correspondence would only be considered if there is an in-kind production from NS. You confirmed that an in-kind production of FDA correspondence should be done promptly. You also confirmed that you will produce by the end of February those NDA sections we requested in our last letter dated January 30, 2023, except for the manufacturing section (which you agreed to consider as discussed below). Please produce those documents without further delay.

**Financial Records:** You wanted us to confirm that the produced financial summary spreadsheet contains “all” units, sold and/or otherwise distributed. Regarding ASP, we explained that you have our total revenue and profits numbers already and there is no need for this type of breakdown. You countered that your request for ASP reports is to distinguish and understand where the units are going to, e.g. commercial drug vs. other drug, for infringement and damages purposes. We committed to following up with the client given your newly explained reasoning. No further agreement was made.

**Exondys 51 Documents:** Commensurate with what we proposed in our last letter, Sarepta has offered to provide sales figures for the subset of patients cross-eligible for Vyondys and Exondys (i.e., exon 52 deletion patients). You further requested “contextual data,” such as what percentage of total Exondys sales that go to the dual product population. We objected to such additional data as being too easy to extrapolate other significant Exondys data. Nevertheless, both sides committed to



exploring what potentially acceptable “contextualizing” data may be provided. We look forward to hearing from you on this issue.

**Manufacturing Documents:** We explained that NS’s manufacturing documents are relevant to NS’s doctrine of equivalents theory in addition to secondary considerations and nexus. We further explained that Sarepta is entitled to discovery of the viltolarsen manufacturing documents/information given NS’s DOE position that the difference to the claimed manufacturing steps of the ’322 patent is insubstantial. You agreed to reconsider in light of this reasoning. We look forward to hearing from you on this issue.

Sincerely,

Yoonhee

**Yoonhee Kim**

Attorney at Law

Finnegan, Henderson, Farabow, Garrett & Dunner, LLP

901 New York Avenue, NW, Washington, DC 20001-4413

202.408.4214 | fax: 202.408.4400 | [yoонhee.kim@finnegan.com](mailto:yoонhee.kim@finnegan.com) | [www.finnegan.com](http://www.finnegan.com)

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**From:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>

**Sent:** Wednesday, February 8, 2023 9:06 AM

**To:** Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>; Clay, Aaron <[Aaron.Clay@finnegan.com](mailto:Aaron.Clay@finnegan.com)>

**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>; Blumenfeld, Jack <[JBlumenfeld@morrisnichols.com](mailto:JBlumenfeld@morrisnichols.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; Chard, Beth Ann <[BChard@morrisnichols.com](mailto:BChard@morrisnichols.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Kozikowski, John <[John.Kozikowski@finnegan.com](mailto:John.Kozikowski@finnegan.com)>; Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>; Dellinger, Megan E. <[mdellinger@morrisnichols.com](mailto:mdellinger@morrisnichols.com)>; Clark, Cameron <[cclark@morrisnichols.com](mailto:cclark@morrisnichols.com)>

**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

**EXTERNAL Email:**

Hi Yoonhee,

Please use the dial-in below:

Dial-In Number: +1-404-410-4502

Conference Code: 661538199

iPhone Friendly Dial-In: +1-404-410-4502, 661538199#

Best,

Mike

**Michael T. Sikora**

**Morgan, Lewis & Bockius LLP**



110 North Wacker Drive | Chicago, IL 60606-1511  
Direct: +1.312.324.1482 | Main: +1.312.324.1000 | Fax: +1.312.324.1001  
[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com) | [www.morganlewis.com](http://www.morganlewis.com)  
Assistant: Deborah McCaskill-Walton | +1.312.324.2581 | [deborah.mccaskill-walton@morganlewis.com](mailto:deborah.mccaskill-walton@morganlewis.com)

---

**From:** Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>

**Sent:** Monday, February 6, 2023 4:41 PM

**To:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>; Clay, Aaron <[Aaron.Clay@finnegan.com](mailto:Aaron.Clay@finnegan.com)>

**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>; Blumenfeld, Jack <[JBlumenfeld@morrisnichols.com](mailto:JBlumenfeld@morrisnichols.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; Chard, Beth Ann <[BChard@morrisnichols.com](mailto:BChard@morrisnichols.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Kozikowski, John <[John.Kozikowski@finnegan.com](mailto:John.Kozikowski@finnegan.com)>; Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>; Dellinger, Megan E. <[mdellinger@morrisnichols.com](mailto:mdellinger@morrisnichols.com)>; Clark, Cameron <[cclark@morrisnichols.com](mailto:cclark@morrisnichols.com)>

**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

[EXTERNAL EMAIL]

Mike,

We are available on Wednesday (2/8) at 10 ET. Please circulate a dial-in. Thank you.

Best regards,  
Yoonhee

**Yoonhee Kim**

Attorney at Law

Finnegan, Henderson, Farabow, Garrett & Dunner, LLP  
901 New York Avenue, NW, Washington, DC 20001-4413  
202.408.4214 | fax: 202.408.4400 | [yunhee.kim@finnegan.com](mailto:yunhee.kim@finnegan.com) | [www.finnegan.com](http://www.finnegan.com)

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**From:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>

**Sent:** Monday, February 6, 2023 8:33 AM

**To:** Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>; Clay, Aaron <[Aaron.Clay@finnegan.com](mailto:Aaron.Clay@finnegan.com)>

**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>; Blumenfeld, Jack <[JBlumenfeld@morrisnichols.com](mailto:JBlumenfeld@morrisnichols.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; Chard, Beth Ann <[BChard@morrisnichols.com](mailto:BChard@morrisnichols.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Kozikowski, John <[John.Kozikowski@finnegan.com](mailto:John.Kozikowski@finnegan.com)>; Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>; Dellinger, Megan E. <[mdellinger@morrisnichols.com](mailto:mdellinger@morrisnichols.com)>

**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

**EXTERNAL Email:**

Hi Yoonhee,

We're unable to make a call today work. Are you available on Wednesday at 10 ET?

Best,

Mike

**Michael T. Sikora**

**Morgan, Lewis & Bockius LLP**

110 North Wacker Drive | Chicago, IL 60606-1511

Direct: +1.312.324.1482 | Main: +1.312.324.1000 | Fax: +1.312.324.1001

[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com) | [www.morganlewis.com](http://www.morganlewis.com)

Assistant: Deborah McCaskill-Walton | +1.312.324.2581 | [deborah.mccaskill-walton@morganlewis.com](mailto:deborah.mccaskill-walton@morganlewis.com)

---

**From:** Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>

**Sent:** Wednesday, February 1, 2023 11:01 AM

**To:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>; Clay, Aaron <[Aaron.Clay@finnegan.com](mailto:Aaron.Clay@finnegan.com)>

**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>; Blumenfeld, Jack <[JBlumenfeld@morrisnichols.com](mailto:JBlumenfeld@morrisnichols.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; Chard, Beth Ann <[BChard@morrisnichols.com](mailto:BChard@morrisnichols.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Kozikowski, John <[John.Kozikowski@finnegan.com](mailto:John.Kozikowski@finnegan.com)>; Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>; Dellinger, Megan E. <[mdellinger@morrisnichols.com](mailto:mdellinger@morrisnichols.com)>

**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

[EXTERNAL EMAIL]

Mike,

Thank you for providing your availability. While we are not available on Thursday, we are available to discuss the outstanding discovery issues on Monday, February 6, at 10 AM (ET). Please let us know if that time works for you and your team.

Best regards,  
Yoonhee

**Yoonhee Kim**

**Attorney at Law**

Finnegan, Henderson, Farabow, Garrett & Dunner, LLP

901 New York Avenue, NW, Washington, DC 20001-4413

202.408.4214 | fax: 202.408.4400 | [yunhee.kim@finnegan.com](mailto:yunhee.kim@finnegan.com) | [www.finnegan.com](http://www.finnegan.com)

---

**From:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>

**Sent:** Tuesday, January 31, 2023 11:08 AM

**To:** Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>; Clay, Aaron <[Aaron.Clay@finnegan.com](mailto:Aaron.Clay@finnegan.com)>

**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>; Blumenfeld, Jack <[JBlumenfeld@morrisnichols.com](mailto:JBlumenfeld@morrisnichols.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lee, Yoonjin

<[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; Chard, Beth Ann <[BChard@morrisnichols.com](mailto:BChard@morrisnichols.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Kozikowski, John <[John.Kozikowski@finnegan.com](mailto:John.Kozikowski@finnegan.com)>; Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>

**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

**EXTERNAL Email:**

Yoonhee,

Is your team available to meet-and-confer Thursday (2/2) at 10 ET / 9 CT?

Best,

Mike

**Michael T. Sikora**

**Morgan, Lewis & Bockius LLP**

110 North Wacker Drive | Chicago, IL 60606-1511

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[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com) | [www.morganlewis.com](http://www.morganlewis.com)

Assistant: Deborah McCaskill-Walton | +1.312.324.2581 | [deborah.mccaskill-walton@morganlewis.com](mailto:deborah.mccaskill-walton@morganlewis.com)

---

**From:** Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>

**Sent:** Monday, January 30, 2023 3:44 PM

**To:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>; Clay, Aaron <[Aaron.Clay@finnegan.com](mailto:Aaron.Clay@finnegan.com)>

**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>; Blumenfeld, Jack

<[JBlumenfeld@morrisnichols.com](mailto:JBlumenfeld@morrisnichols.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lee, Yoonjin

<[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; Chard, Beth Ann

<[BChard@morrisnichols.com](mailto:BChard@morrisnichols.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>;

Kozikowski, John <[John.Kozikowski@finnegan.com](mailto:John.Kozikowski@finnegan.com)>; Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>;

Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>

**Subject:** RE: Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

[EXTERNAL EMAIL]

Mike,

Please see the attached response to your January 17, 2023, letter regarding discovery.

Regards,

Yoonhee

**Yoonhee Kim**

**Attorney at Law**

Finnegan, Henderson, Farabow, Garrett & Dunner, LLP

901 New York Avenue, NW, Washington, DC 20001-4413

202.408.4214 | fax: 202.408.4400 | [yoонhee.kim@finnegan.com](mailto:yoонhee.kim@finnegan.com) | [www.finnegan.com](http://www.finnegan.com)

---

**From:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>  
**Sent:** Tuesday, January 17, 2023 6:31 PM  
**To:** Clay, Aaron <[Aaron.Clay@finnegan.com](mailto:Aaron.Clay@finnegan.com)>  
**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>; Blumenfeld, Jack <[JBlumenfeld@morrisnichols.com](mailto:JBlumenfeld@morrisnichols.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; Chard, Beth Ann <[BChard@morrisnichols.com](mailto:BChard@morrisnichols.com)>; Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Kozikowski, John <[John.Kozikowski@finnegan.com](mailto:John.Kozikowski@finnegan.com)>; Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>  
**Subject:** Nippon Shinyaku Co. Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-GBW (D. Del.)

**EXTERNAL Email:**

Aaron,

Please see the attached letter.

Best,

Mike

**Michael T. Sikora**

**Morgan, Lewis & Bockius LLP**

110 North Wacker Drive | Chicago, IL 60606-1511

Direct: +1.312.324.1482 | Main: +1.312.324.1000 | Fax: +1.312.324.1001

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# EXHIBIT L



**From:** [O'Quinn, Ryan](#)  
**To:** [Sikora, Michael T.](#); [Lipton, Alissa](#); [NS District Court](#)  
**Cc:** [Lipsey, Charles](#); [Raich, William](#); [Flibbert, Michael](#); [McCorquindale, J. Derek](#); [Lee, Yoonjin](#); [Kim, Yoonhee](#); [jblumenfeld@morrisnichols.com](#); [Dellinger, Megan E.](#)  
**Subject:** RE: Nippon Shinyaku, Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-LPS – Document Production  
**Date:** Wednesday, May 24, 2023 3:12:56 PM  
**Attachments:** [image002.png](#)  
[image003.png](#)

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Mike,

We write further to the parties' May 22, 2023 meet and confer. As discussed during the meet and confer, we will respond separately regarding NS's request for non-golodirsén documents relating to work performed by Sazani and Kole on exon 53 oligos.

Regarding ex-US sales data, we restated our views that this data is outside the scope of this case, and that *WesternGeco* does not provide an unfettered right to extraterritorial damages. Nevertheless, solely to resolve the dispute and avoid burdening the Court and without waiving its objections including relevance, Sarepta can agree to re-produce the 116 weekly sales reports that were originally produced in Sarepta's production volume no. 12 by the end of this week in a form that un-redacts sales figures for units manufactured in the United States that are sold or disposed of outside the United States, contingent on NS either producing in-kind information (i.e. sales figures for any Viltepso exported from the US to other countries) or a written confirmation there are no such units. When you discussed production of data down to the "customer" level, we reiterated that aggregated sales data would be sufficient and proportional to the needs of the case. You then stated that this exchange would settle the dispute on the assumption that it provides detail necessary to perform a reasonable royalty analysis. Please indicate whether NS will be producing any sales data pursuant to this agreement, or provide NS's written statement.

The parties next discussed the issues relating to DMD licenses, and we will let the forthcoming briefing on that impasse speak for itself.   


On switching data, we understood the parties to have agreed to mutually provide data sufficient to show their tracking of switching among Exondys/Vyondys and Viltepso, tentatively by the end of May. You suggested that NS has already produced data that would be subject to this compromise, for example NS0074851 in NS's production volume no. 9 in April. We observed that this information was informal and unorganized. Please confirm that NS will collect and produce documents sufficient to show patient switching data that it maintains and/or monitors for these drugs. Additionally, we inquired regarding NS's documents concerning exon 52-deletion patients. You stated that you believed that all such documents from NS had been collected and produced, but that you would re-review the ESI to confirm. Please identify the exon 52-related documents that have been produced, and produce any additional documents relating to exon 52 by the end of May.

Finally, as discussed previously, Sarepta has maintained its request for documents related to Exon 53 oligos dated after the December 2013 cut-off date imposed by NS. During the May 22, 2023 meet

and confer, NS offered to potentially resolve this dispute by providing laboratory notebooks and data related to the experiments performed by or at the direction of NS in connection with (1) prosecution of one or more of the following European Patent Applications: EP10004274.6 (published as EP2206781A1), EP11821996.3 (published as EP2612917A1), EP15199455.5 (published as EP3018211A1), EP19169673.1 (published as EP3543341A1), and EP19188572.2 (published as EP3581655A1); or (2) proceedings involving any patent that issued therefrom. If NS were to promptly provide such documents, Sarepta would be willing to agree that this dispute has been resolved. Please confirm that NS has the same understanding. Alternatively, please confirm that the parties are at an impasse on this issue.

With best regards,  
Ryan

**Ryan P. O'Quinn, Ph.D.**  
Partner

Finnegan, Henderson, Farabow, Garrett & Dunner, LLP  
1875 Explorer Street, Suite 800, Reston, VA 20190-6023  
571.203.2426 | fax: 202.408.4400 | [ryan.o'quinn@finnegan.com](mailto:ryan.o'quinn@finnegan.com) | [www.finnegan.com](http://www.finnegan.com)

**FINNEGAN**

---

**From:** O'Quinn, Ryan

**Sent:** Monday, May 22, 2023 4:47 PM

**To:** Sikora, Michael T. <michael.sikora@morganlewis.com>; Lipton, Alissa  
<Alissa.Lipton@finnegan.com>; NS District Court <NSDistrictCourt@morganlewis.com>

**Cc:** Lipsey, Charles <charles.lipsey@finnegan.com>; Raich, William <William.Raich@finnegan.com>;  
Flibbert, Michael <michael.flibbert@finnegan.com>; McCorquindale, J. Derek  
<Derek.McCorquindale@finnegan.com>; Lee, Yoonjin <Yoonjin.Lee@finnegan.com>; Kim, Yoonhee  
<Yoonhee.Kim@finnegan.com>; jblumenfeld@morrisnichols.com; Dellinger, Megan E.  
<mdellinger@morrisnichols.com>

**Subject:** RE: Nippon Shinyaku, Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-LPS – Document Production

Mike,

Thanks to you and your team for your time on the meet and confer this morning. We will send further detail about the parties' discussions of other issues under separate cover.

With regard to the draft joint letter to the Court, Sarepta's proposed redline to your Friday draft is attached, adding reference to this morning's call and narrowing the issues in dispute to the single issue of DMD-related licenses. Please let us know if NS has any further edits before any filing with sufficient time to review with our client.





With best regards,

Ryan

**Ryan P. O'Quinn, Ph.D.**

Partner

Finnegan, Henderson, Farabow, Garrett & Dunner, LLP

1875 Explorer Street, Suite 800, Reston, VA 20190-6023

571.203.2426 | fax: 202.408.4400 | [ryan.o'quinn@finnegan.com](mailto:ryan.o'quinn@finnegan.com) | [www.finnegan.com](http://www.finnegan.com)

**FINNEGAN**

---

**From:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>

**Sent:** Sunday, May 21, 2023 4:01 PM

**To:** O'Quinn, Ryan <[Ryan.O'Quinn@finnegan.com](mailto:Ryan.O'Quinn@finnegan.com)>; Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>; NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>

**Cc:** Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>; [jblumenfeld@morrisnichols.com](mailto:jblumenfeld@morrisnichols.com); Dellinger, Megan E. <[mdellinger@morrisnichols.com](mailto:mdellinger@morrisnichols.com)>

**Subject:** RE: Nippon Shinyaku, Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-LPS – Document Production



External email >

**EXTERNAL Email:**

Ryan,

A dial-in for tomorrow morning is below.

Dial-In Number: +1-404-410-4502

Conference Code: 661538199

iPhone Friendly Dial-In: +1-404-410-4502, 661538199#

Best,

Mike

**Michael T. Sikora**

**Morgan, Lewis & Bockius LLP**

110 North Wacker Drive | Chicago, IL 60606-1511

Direct: +1.312.324.1482 | Main: +1.312.324.1000 | Cell: +1.651.233.8640 | Fax: +1.312.324.1001

[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com) | [www.morganlewis.com](http://www.morganlewis.com)

---

**From:** O'Quinn, Ryan <Ryan.O'Quinn@finnegan.com>

**Sent:** Saturday, May 20, 2023 2:45 PM

**To:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>; Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>; NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>

**Cc:** Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>; [jblumenfeld@morrisnichols.com](mailto:jblumenfeld@morrisnichols.com); Dellinger, Megan E. <[mdellinger@morrisnichols.com](mailto:mdellinger@morrisnichols.com)>

**Subject:** RE: Nippon Shinyaku, Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-LPS – Document Production

[EXTERNAL EMAIL]

Mike,

You're correct that we discussed the parties' mutual discovery issues on the April 14th meet and confer. You're incorrect, however, that the only thing required in the intervening five weeks was for "Sarepta to reconsider its positions." We asked for elaboration and information from NS on the April 14th call regarding multiple different issues which you agreed to investigate, and have received virtually none of what we requested. It is unreasonable and untenable for NS to now re-emerge five weeks closer to the discovery deadlines and suddenly demand unilateral production from Sarepta with no further collaboration or discussion about how NS will also supplement its deficient production. Discovery, particularly with respect to damages, flows both ways in this case. We have seen no indication from NS that it intends to make a production commensurate with what it demands from Sarepta.

We would like to meet and confer on Monday morning in the spirit of that collaboration. We are available at 8:30 AM ET. Please provide dial-in information.

As to NS's document production, we disagree that these are "separate issues" or that we can wait for NS to respond in "due course." This only reinforces the illusory nature of NS's other four artificial "impasses." We look forward to receiving a status update on Monday regarding NS's document production as well as Sarepta's compromise proposal on the exon 53 skipping documents that NS has thus far refused to produce. As we noted during the April 14th meet and confer, the vast majority of documents produced by NS have been in Japanese. Particularly in view of this circumstance, it is unreasonable to expect the deposition schedule to remain on track if NS does not promptly complete its document production.

We look forward to speaking with you.

With best regards,  
Ryan

**Ryan P. O'Quinn, Ph.D.**

Partner

Finnegan, Henderson, Farabow, Garrett & Dunner, LLP

1875 Explorer Street, Suite 800, Reston, VA 20190-6023

571.203.2426 | fax: 202.408.4400 | [ryan.o'quinn@finnegan.com](mailto:ryan.o'quinn@finnegan.com) | [www.finnegan.com](http://www.finnegan.com)

**FINNEGAN**

---

**From:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>

**Sent:** Friday, May 19, 2023 6:18 PM

**To:** Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>; NS District Court  
<[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>

**Cc:** Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>;  
Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; McCorquindale, J. Derek  
<[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Kim, Yoonhee  
<[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>; [jblumenfeld@morrisnichols.com](mailto:jblumenfeld@morrisnichols.com); Dellinger, Megan E.  
<[mdellinger@morrisnichols.com](mailto:mdellinger@morrisnichols.com)>

**Subject:** RE: Nippon Shinyaku, Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-LPS – Document Production

**EXTERNAL Email:**

Alissa,

NS has done anything but “rush” to the Court here. As your email noted, we discussed NS’s arguments regarding why the requested discovery is appropriate on the April 14<sup>th</sup> meet-and-confer, and have since provided ample time for Sarepta to reconsider its positions. Sarepta clearly continues to disagree with NS’s arguments, which we still contend more-than-justify the requested document production. There is no requirement that we serially invent new arguments for Sarepta’s consideration until it finally is persuaded to produce documents.

Even in your emails, Sarepta tellingly makes *no offers* to produce any of the requested information about which we are at an impasse. We are not willing to further delay resolution of these issues. That said, in the interests of cooperation, we are willing to meet and confer once more on these four issues anytime this weekend, or before 11am ET on Monday, should Sarepta still believe doing so would be fruitful.

We will respond to the separate issues your email raises (e.g., regarding NS’s document production) in due course.

Best,

Mike

**Michael T. Sikora**

**Morgan, Lewis & Bockius LLP**

110 North Wacker Drive | Chicago, IL 60606-1511

Direct: +1.312.324.1482 | Main: +1.312.324.1000 | Cell: +1.651.233.8640 | Fax: +1.312.324.1001

[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com) | [www.morganlewis.com](http://www.morganlewis.com)

---

**From:** Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>

**Sent:** Friday, May 19, 2023 4:01 PM

**To:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>; NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>

**Cc:** Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>; [jblumenfeld@morrisnichols.com](mailto:jblumenfeld@morrisnichols.com); Dellinger, Megan E. <[mdellinger@morrisnichols.com](mailto:mdellinger@morrisnichols.com)>

**Subject:** RE: Nippon Shinyaku, Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-LPS – Document Production

[EXTERNAL EMAIL]

Mike,

We write in response to your email of 11:00 p.m. last night. In NS's rush to go to the court over disputes that are not at an impasse, your email fails to even acknowledge the parties' discussion during the last meet and confer held on April 14, 2023. Despite Sarepta's specific requests during the meet and confer that NS provide information regarding the basis for its requests and/or its own document production on the topics you raise, we have heard nothing from NS prior to your unilateral statement a few days ago that NS has decided that the parties are at an impasse on these topics. To be clear, we reiterate our statement in our email from Wednesday that the parties are not at an impasse regarding any of the four topics raised in your email.

First, regarding "[s]ales figures for Vyondys53 units manufactured in the United States that Sarepta ultimately exported/disposed of internationally," we asked you during and after the April 14th meet and confer what case law NS possessed supporting its effort to increase the scope of damages discovery in this case late in the fact discovery period. Your May 15th email—more than a month later—provided nothing but the *WesternGeco* case that we discussed (and that Sarepta distinguished) during the meet and confer. As we discussed on the meet and confer, *WesternGeco* does not stand for the broad proposition that a patentee is entitled to extraterritorial foreign damages for any and all acts of domestic patent infringement. *WesternGeco* is a § 271(f) case. NS has not alleged a § 271(f) infringement theory in this case, nor can it. In other words, **nothing** was done on NS's part in the last 31 days to move the ball forward on resolving this issue. The previous month could have been spent discussing the details of a possible mutual exchange of foreign sales data, but instead, NS now simply wants to manufacture an impasse and heap undue burden onto the parties and onto the Court. That said, we remain willing to meet and confer to avoid this burden.

Secondly, with respect to "license agreements relating to DMD therapies beyond solely exon-skipping therapies," NS still has provided us with no reason why this information is relevant. NS has

notably not offered to provide any of its own non-exon-skipping licenses or agreements, which lends credence to the notion that this is nothing more than an attempted fishing expedition into Sarepta's sensitive business information by a direct competitor. Nevertheless, we are willing to try to resolve this issue without including the Court.

With respect to patient-switching data and exon 52 deletion patients, Sarepta's produced spreadsheet provided all of the information that NS requested and that NS needs. Claims data for these therapies is publicly available if NS wishes to further analyze patient metrics relating to its own products. Notably, we asked you during the April 14th meet and confer whether NS would be producing its own internal switching data as part of a mutual in-kind exchange. To date, we have not seen any such production or heard any updates whatsoever from you in that regard, leaving this issue open. If NS has already produced any documents that it believes constitutes switching data, please provide Bates numbers. Regardless, we remain available to discuss how to mutually resolve these issues.

To be clear, Sarepta is not unilaterally refusing to produce documents from these three damages-related categories in any form, and never has. We were waiting for more information from NS to facilitate a further discussion towards resolving these disputes. Instead, we received two messages from you insisting we are at an impasse. We disagree and continue to believe there is room to resolve these issues bilaterally, which we hope you will join us in doing next week.

Finally, when we discussed on the last meet and confer NS's request for documents related to Sazani and Kole's work related to exon 53 skipping oligos (but not related to golodirsen), we pointed out that NS has failed to produce *any* documents of the sort that it is now pushing for. As NS is aware, and as we have reiterated multiple times, Sarepta has maintained its request that NS produce documents related to testing of exon 53 oligos regardless of the date of these documents (NS arbitrarily imposed a December 2013 date cut off on its production of any documents). In order to resolve the parties' dispute regarding NS's deficient production of documents related to exon 53 skipping data, and to allow Sarepta to properly consider NS's request for non-golodirsen documents from Sazani and Kole, please confirm the following:

NS agrees with Sarepta's proposal that production of non-privileged documents related to exon 53 skipping oligos, dated prior to the filing of the complaint, will complete the parties' discovery obligations with respect to production of non-damages documents in this case (along with documents that describe the work leading to the filing of the patents in suit, as described below):

1. Documents sufficient to show analyses of western blot methodology for dystrophin
2. Documents sufficient to show any non-clinical testing of PMOs and 2'-O-Me's targeting +36+60, +36+56, +35+59, and/or +32+56
3. Documents sufficient to show any communications with the UWA Inventors
4. Documents sufficient to show any research performed by NCNP related to exon-53 skipping PMOs and 2'-O-Me's

Additionally, in response to your request during the April 14, 2023, meet and confer, we confirm that Sarepta has produced the following development documents:

1. Sarepta has produced all of the UWA laboratory notebooks that could be located related to

the research leading to the inventions claimed in the UWA patents as well as additional laboratory notebooks related to exon 53 research. The more than 60 UWA laboratory notebooks produced in total are sufficient to show the conception and reduction to practice of the inventions claimed in the UWA Patents.

2. Sarepta has produced relevant portions of the NDA for golodirsén.
3. Sarepta has produced a research report memorializing [REDACTED], as well as the Sarepta laboratory notebooks cited therein.
4. Sarepta has produced emails sufficient to show the events surrounding [REDACTED]

Please similarly confirm that it is NS's understanding that it has produced or previously agreed to produce the following development documents: [advise as to the status of the rolling productions]

1. NS has produced NS laboratory notebooks and weekly reports relating to the work leading to the filing of the NS Patents. NS has indicated that no NCNP notebooks relating to the work that led to the filing of the NS Patents have been located and has not otherwise produced documents from NCNP. NS will investigate whether there are additional NS or any NCNP laboratory notebooks or other paper documents relating to the work that led to the filing of the NS Patents and produce those documents if they are identified.
2. NS has produced relevant portions of the NDA for viltolarsén.
3. NS will produce additional relevant electronic documents, including emails and accompanying attachments, based on a search using terms NS previously identified of documents from Watanabe, Satou, and the server associated with NS's Discovery Research Laboratories in Tsukuba.

In addition, based on NS and NCNP's representations during the April 14, 2023 meet and confer, please also confirm that NS and NCNP have performed a reasonable search for and have produced any non-privileged documents resulting from that search in the following categories:

1. Laboratory notebooks or other documents from NCNP related to the work that led to the filing of the NS Patents.
2. Documents from NS related to the work that led to the filing of the NS Patents, in particular, the examples in the NS Patents.

Regards,  
Alissa

**Alissa K. Lipton**  
Partner

Finnegan, Henderson, Farabow, Garrett & Dunner, LLP  
901 New York Avenue, NW  
Washington DC 20001-4413

Two Seaport Lane, 6th Floor  
Boston, MA 02210-2001

617.646.1643 | fax 617.646.1666 | [alissa.lipton@finnegan.com](mailto:alissa.lipton@finnegan.com) | [www.finnegan.com](http://www.finnegan.com)

---

**From:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>

**Sent:** Thursday, May 18, 2023 11:00 PM

**To:** Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>; NS District Court  
<[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>

**Cc:** Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>;  
Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; McCorquindale, J. Derek  
<[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Kim, Yoonhee  
<[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>; [jblumenfeld@morrisnichols.com](mailto:jblumenfeld@morrisnichols.com); Dellinger, Megan E.  
<[mdellinger@morrisnichols.com](mailto:mdellinger@morrisnichols.com)>

**Subject:** RE: Nippon Shinyaku, Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-LPS – Document Production

**EXTERNAL Email:**

Alissa,

NS has been attempting to resolve these particular disputes with Sarepta for at least several months now. After the deadline for substantial document completion alone, the parties have exchanged much correspondence and met-and-conferred at least twice. Although we have resolved other disputes in that time, Sarepta has not budged on its positions regarding any of these four issues.

1. Regarding, sales figures for Vyondys53 units manufactured in the United States that Sarepta ultimately exported/disposed of internationally, Sarepta's position remains the same as that articulated in Yoonhee's email from two months ago. Kim Email (Mar. 20, 2023) ("[Y]our request to remove redactions is directed to ex-US customers and sales information. Sarepta has the right to redact confidential commercial information not relevant to the case under the Protective Order and will not remove these redactions.").
2. Regarding license agreements relating to DMD therapies beyond solely exon-skipping therapies (e.g., Sarepta's licenses for SRP-9001, including an unredacted version of the Roche Agreement), Sarepta's position remains the same as that articulated in Yoonhee's February 21 email. Kim Email (Feb. 21, 2023) ("Sarepta maintains that 'licenses relating to treatments for DMD beyond exon 53-directed oligonucleotides' are not relevant to this case, and will not be produced.").
3. Regarding patient-switching information, Sarepta's position remains the same as that articulated in Yoonhee's email from two months ago. Kim Email (Mar. 20, 2023) (declining NS's proposal and stating that "Sarepta plans to provide documents containing information sufficient to show at least the number of exon 51/exon 53 amenable patients to which it has provided drug, which drug was provided, the weight of the patient, and the start date and status of therapy.").
4. And regarding the exon 53-related experimentation conducted by Peter Sazani and Rysvard

Kole, Sarepta's position remains that articulated at least as early as our February meet-and-confer. *See, e.g.*, Sikora Email (Feb. 10, 2023) (noting Sarepta's position that it would be withholding PPMO experimentation).

Unless Sarepta is prepared to make a full production of these items immediately, we fail to see how the parties are not at an impasse. Fact deposition dates are now being set and NS is being prejudiced in its ability to prepare its case and effectively prepare for depositions without the requested information. If you cannot confirm that full production will be made for each issue by the end of this week (as my Monday email requested), then an impasse exists for which the parties need the court's intervention.

Thank you for noting Judge William's differing procedure on disputes. If Sarepta cannot commit to a date certain for production, we request local counsel's authorization to submit the draft joint letter attached. Of course, to the extent Sarepta can commit to productions on at least some issues, please feel free to propose commensurate revisions eliminating the disputed issue(s).

Best,

Mike

**Michael T. Sikora**

**Morgan, Lewis & Bockius LLP**

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---

**From:** Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>

**Sent:** Wednesday, May 17, 2023 3:45 PM

**To:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>; NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>

**Cc:** Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>; [jblumenfeld@morrisnichols.com](mailto:jblumenfeld@morrisnichols.com); Dellinger, Megan E. <[mdellinger@morrisnichols.com](mailto:mdellinger@morrisnichols.com)>

**Subject:** RE: Nippon Shinyaku, Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-LPS – Document Production

[EXTERNAL EMAIL]

Mike,

We disagree that the parties are at an impasse regarding any of the four topics outlined in your email. Please let us know your availability for a meet and confer on Wednesday, May 24<sup>th</sup> to discuss these topics.

(We also note, should the parties reach an impasse on an issue in the future, that Judge Williams's procedures for discovery disputes, as outlined in Paragraph 4(g) of the Scheduling Order (D.I. 143),



require submitting a joint letter to the court, not a call.)

Regards,  
Alissa

**Alissa K. Lipton**  
Partner

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901 New York Avenue, NW  
Washington DC 20001-4413

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Boston, MA 02210-2001  
617.646.1643 | fax 617.646.1666 | [alissa.lipton@finnegan.com](mailto:alissa.lipton@finnegan.com) | [www.finnegan.com](http://www.finnegan.com)

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**From:** Sikora, Michael T. <[michael.sikora@morganlewis.com](mailto:michael.sikora@morganlewis.com)>  
**Sent:** Monday, May 15, 2023 9:11 PM  
**To:** Lee, Yoonjin <[Yoonjin.Lee@finnegan.com](mailto:Yoonjin.Lee@finnegan.com)>; Dellinger, Megan E. <[mdellinger@morrisnichols.com](mailto:mdellinger@morrisnichols.com)>; Jblumenfeld@morrisnichols.com; Raich, William <[William.Raich@finnegan.com](mailto:William.Raich@finnegan.com)>; Lipton, Alissa <[Alissa.Lipton@finnegan.com](mailto:Alissa.Lipton@finnegan.com)>; Lipsey, Charles <[charles.lipsey@finnegan.com](mailto:charles.lipsey@finnegan.com)>; Flibbert, Michael <[michael.flibbert@finnegan.com](mailto:michael.flibbert@finnegan.com)>; McCorquindale, J. Derek <[Derek.McCorquindale@finnegan.com](mailto:Derek.McCorquindale@finnegan.com)>; Kim, Yoonhee <[Yoonhee.Kim@finnegan.com](mailto:Yoonhee.Kim@finnegan.com)>  
**Cc:** NS District Court <[NSDistrictCourt@morganlewis.com](mailto:NSDistrictCourt@morganlewis.com)>  
**Subject:** Nippon Shinyaku, Ltd. v. Sarepta Therapeutics, Inc., No. 1:21-cv-01015-LPS – Document Production

**EXTERNAL Email:**

Counsel,

Based on our prior meet-and-confer, we understand the parties to be at an impasse as to the following issues. Could you please provide local counsel's availability this Thursday or Friday to call the court to schedule a discovery hearing? Alternatively, if Sarepta agrees to make a full production of the information by the end of this week, please let us know before that call.

5. Sales figures for Vyondys53 units manufactured in the United States that Sarepta ultimately exported/disposed of internationally. NS continues to believe that it is entitled to this information. *See, e.g., WesternGeco LLC v. ION Geophysical Corp.*, 138 S. Ct. 2129, 2138 (2018) (affirming that damages were available for foreign sales because "[t]he conduct in this case that is relevant to that focus clearly occurred in the United States, as it was ION's domestic act of supplying the components that infringed WesternGeco's patents").
6. License agreements relating to DMD therapies beyond solely exon-skipping therapies (*e.g.*, Sarepta's licenses for SRP-9001, including an unredacted version of the Roche Agreement).
7. Documents (beyond the list of patients with an exon 52 deletion Sarepta has provided)

sufficient to show Sarepta's tracking of patient-switching between Exondys51, Vyondys53 and/or Viltepso, and the reasons therefore.

8. Documents sufficient to show the exon 53-related experimentation conducted by Peter Sazani and Rysvard Kole (e.g., as provided in U.S. 2010/0130591 A1).

Best,

Mike

**Michael T. Sikora**

**Morgan, Lewis & Bockius LLP**

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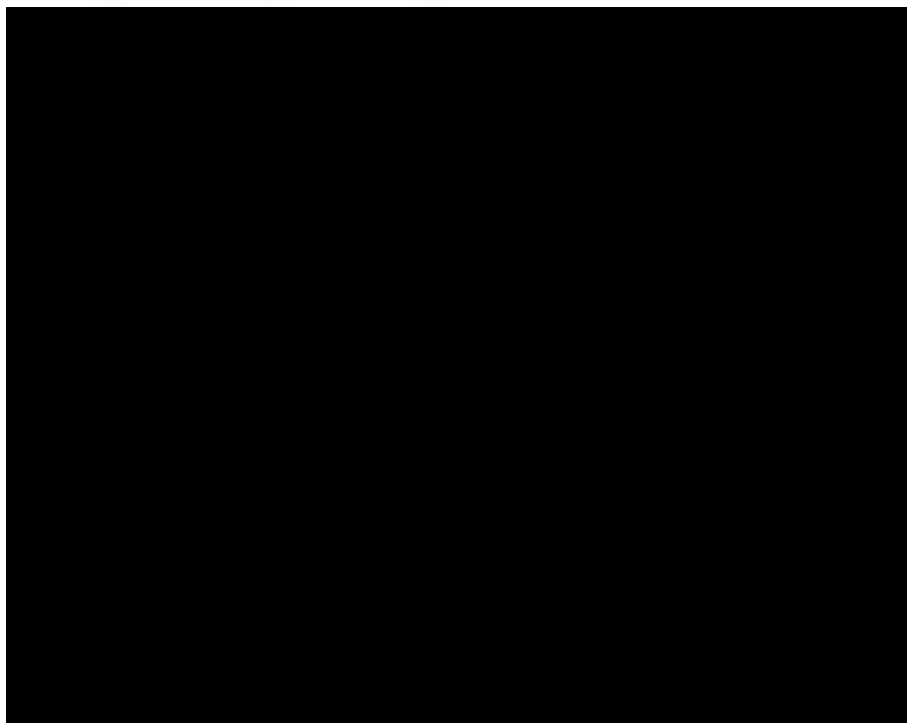
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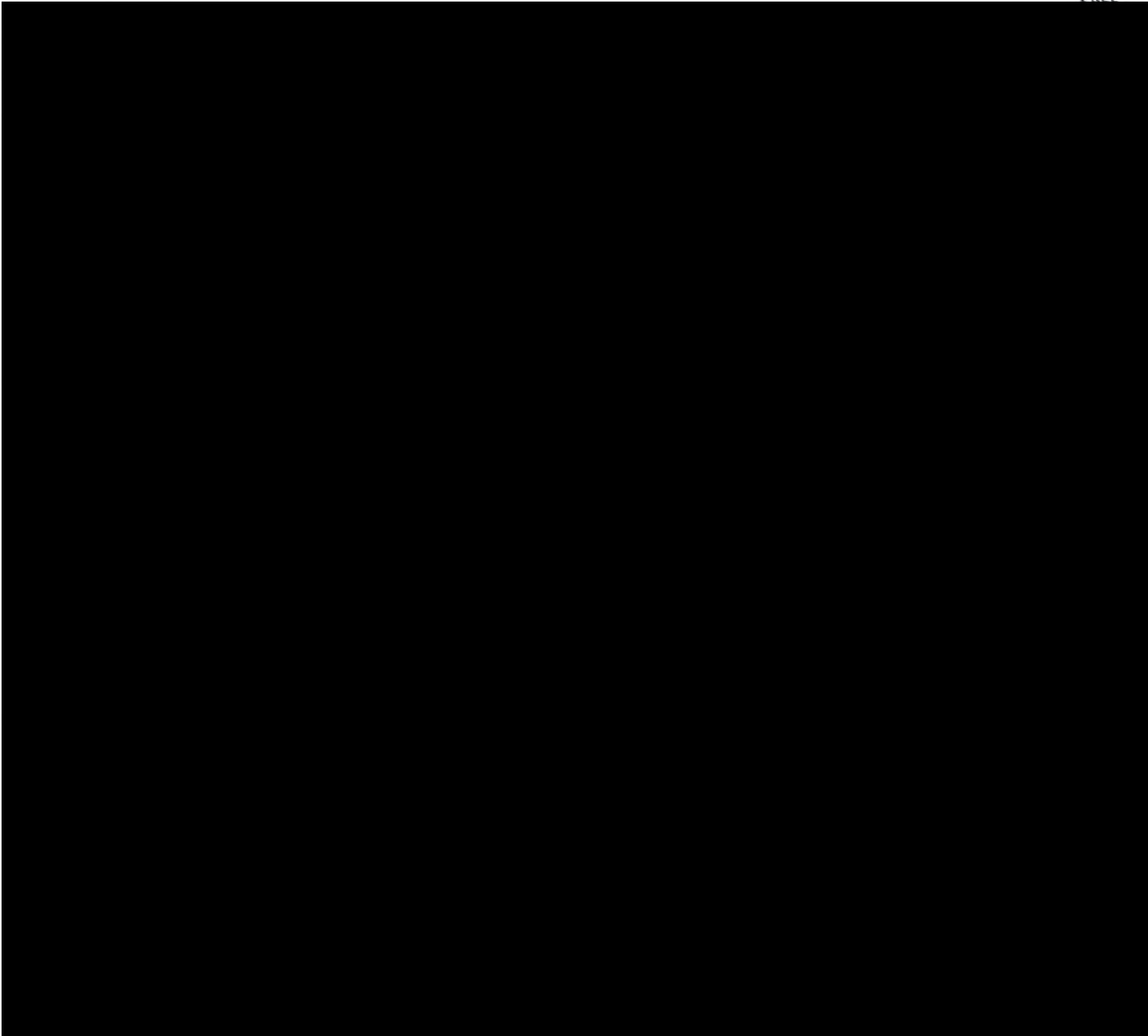
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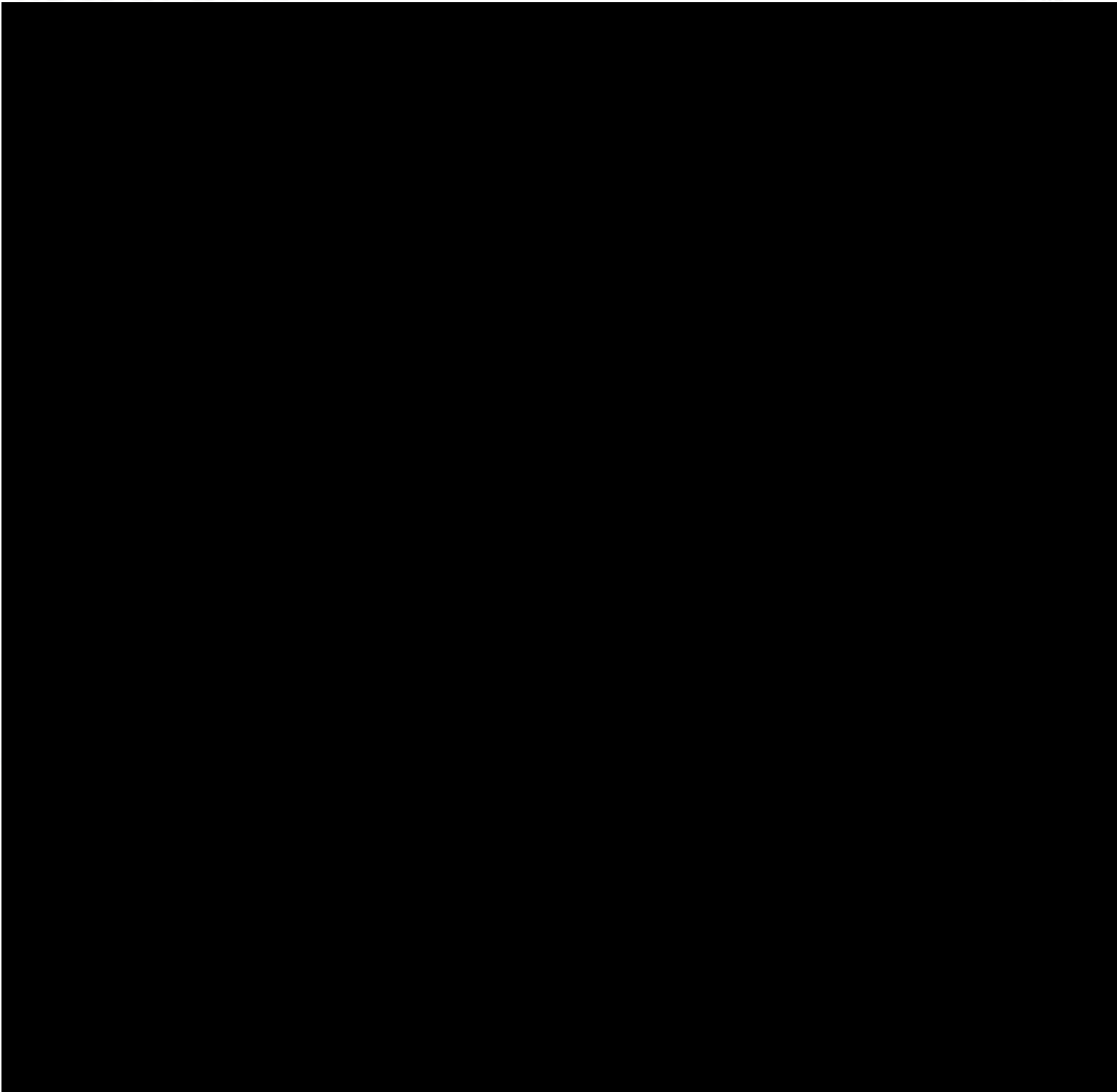
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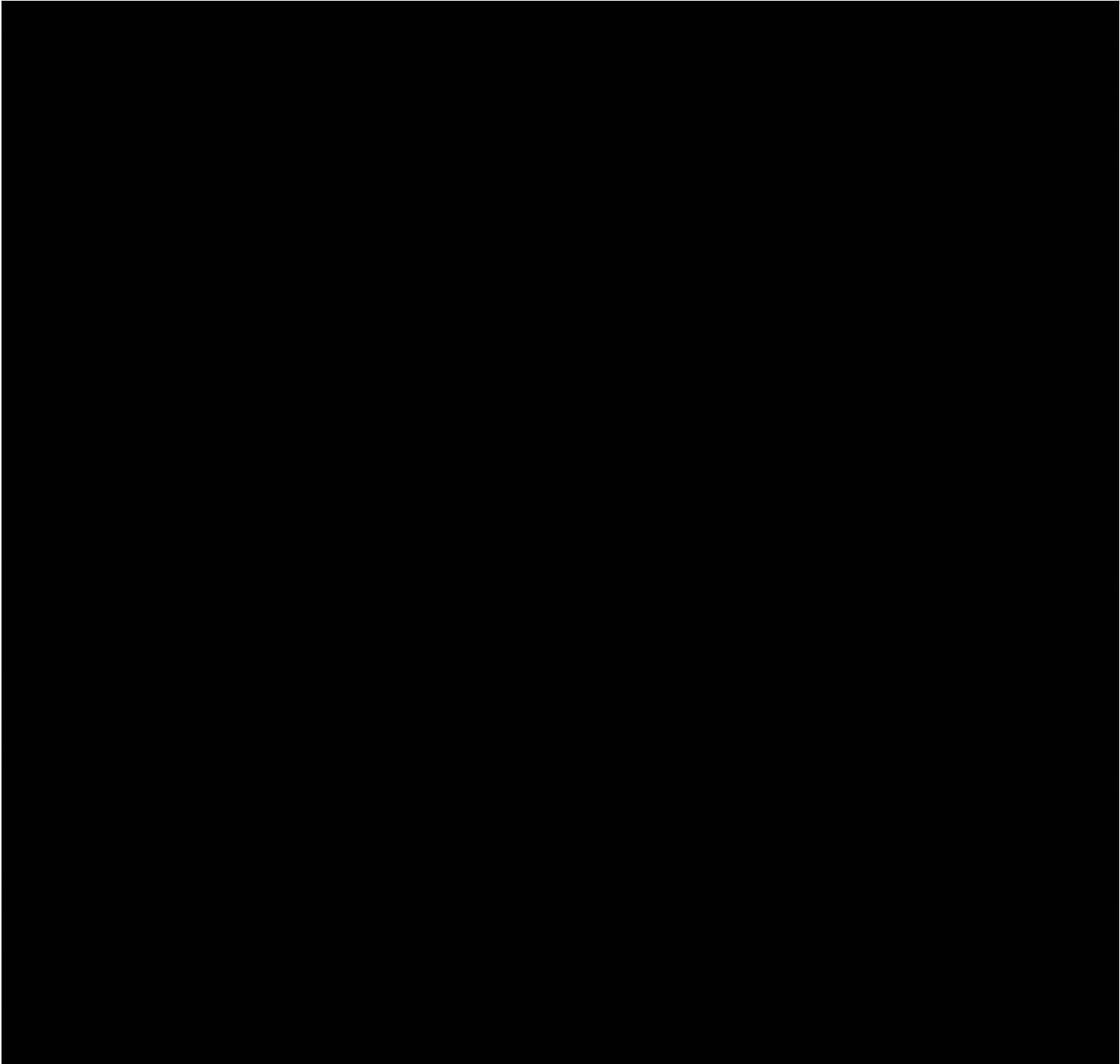
CERTAIN CONFIDENTIAL INFORMATION CONTAINED IN THIS DOCUMENT, MARKED BY [\*\*], HAS BEEN OMITTED BECAUSE IT IS NOT MATERIAL AND WOULD LIKELY CAUSE COMPETITIVE HARM TO SAREPTA THERAPEUTICS, INC. IF PUBLICLY DISCLOSED.

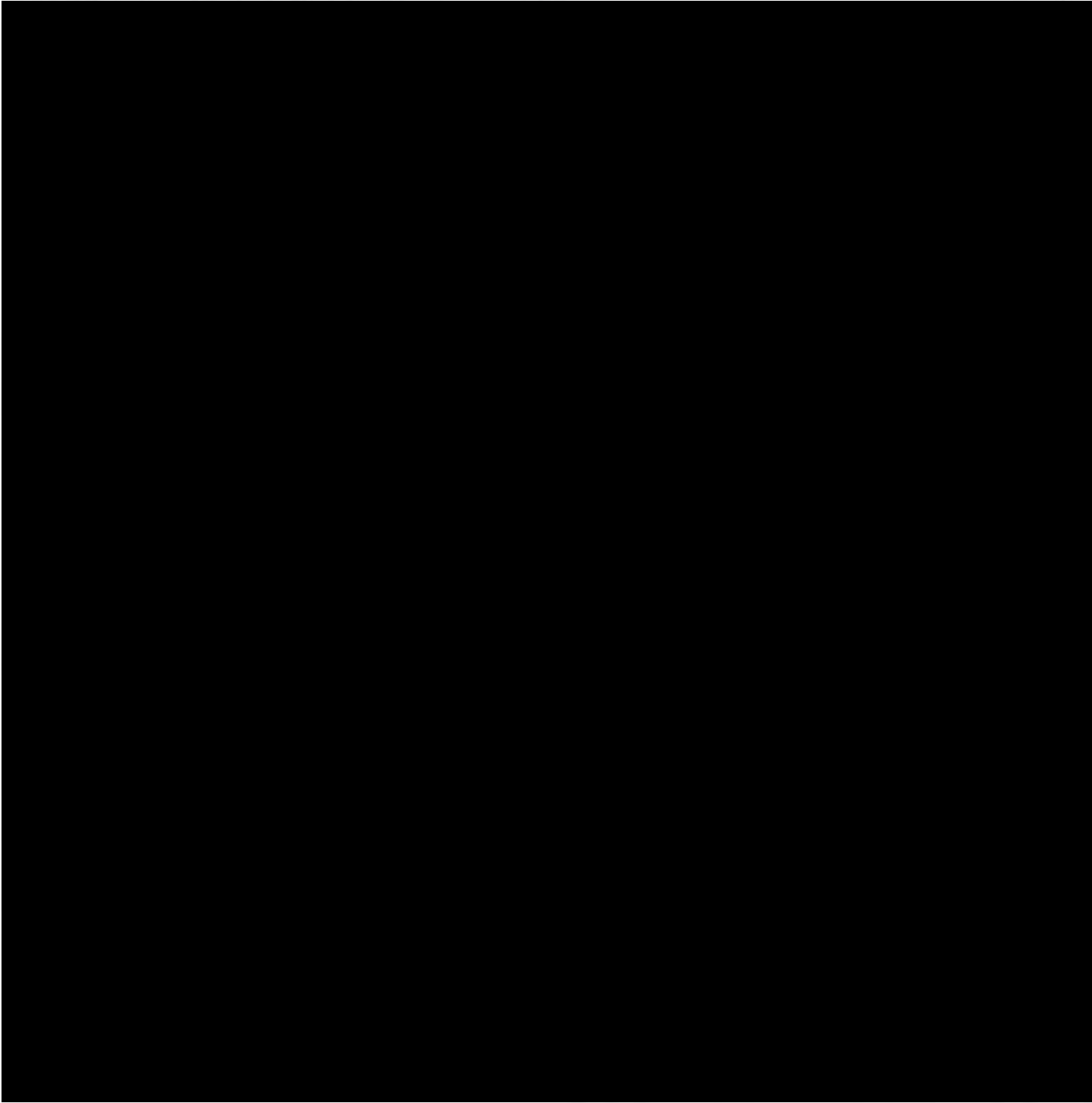


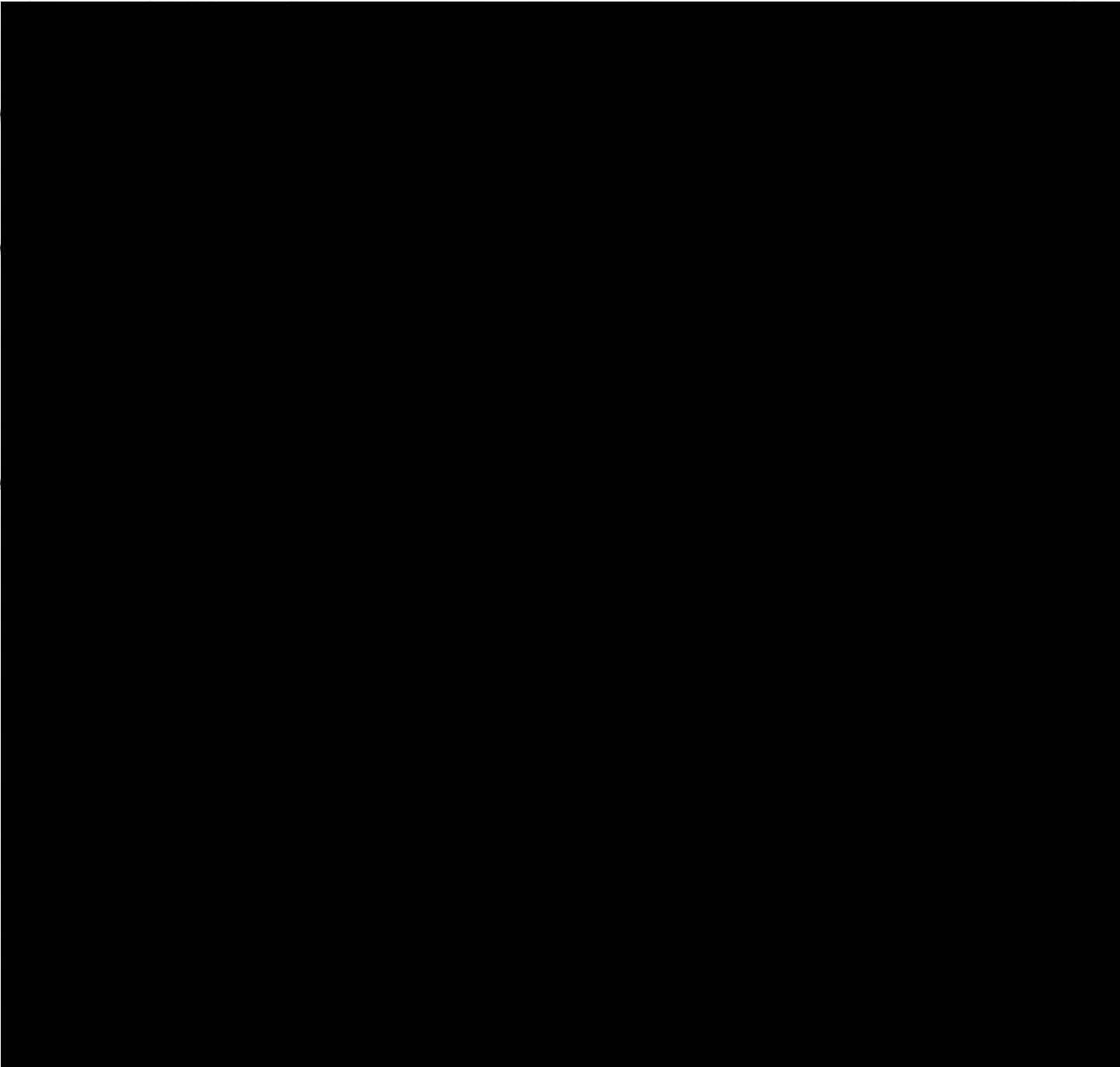
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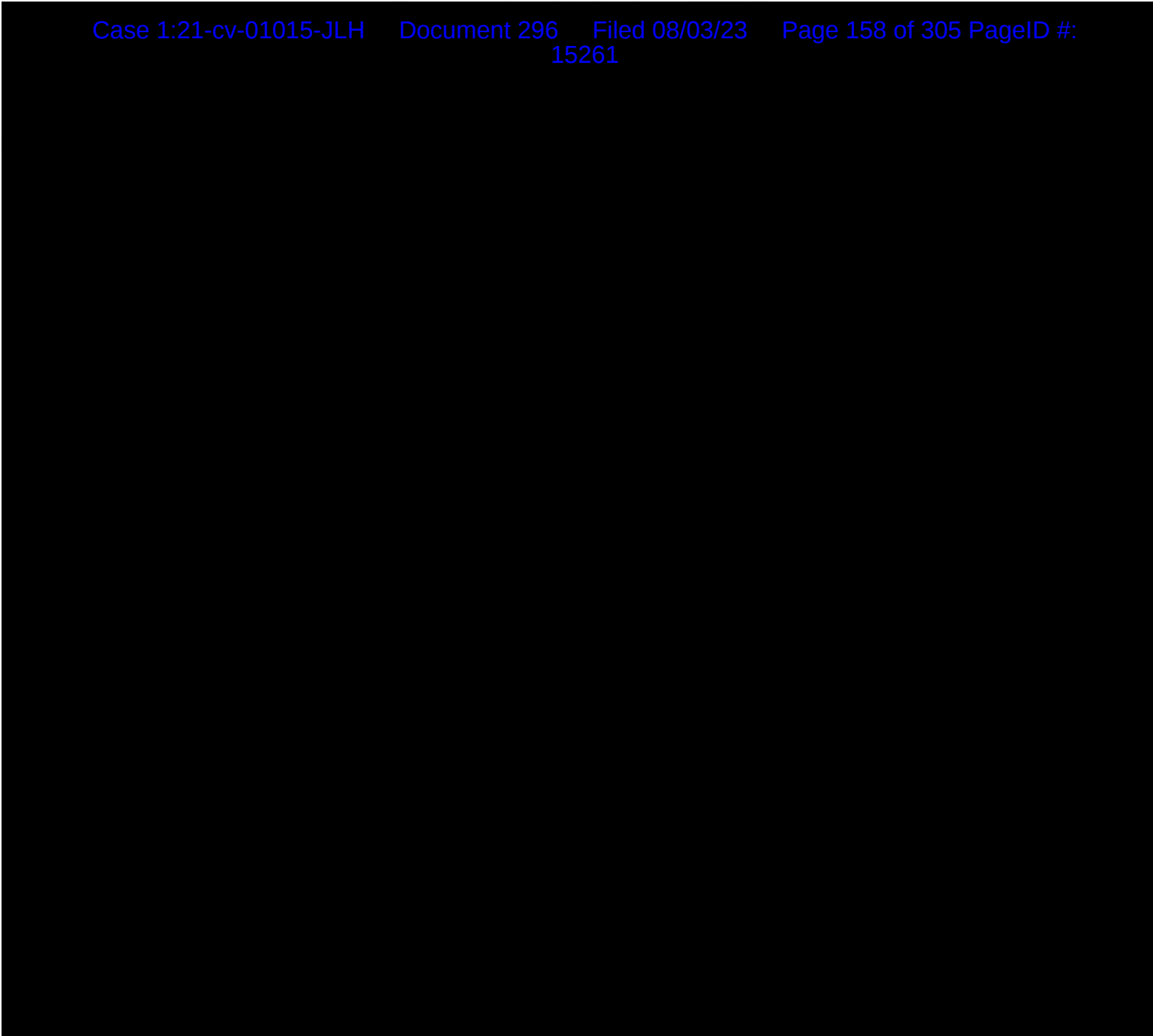


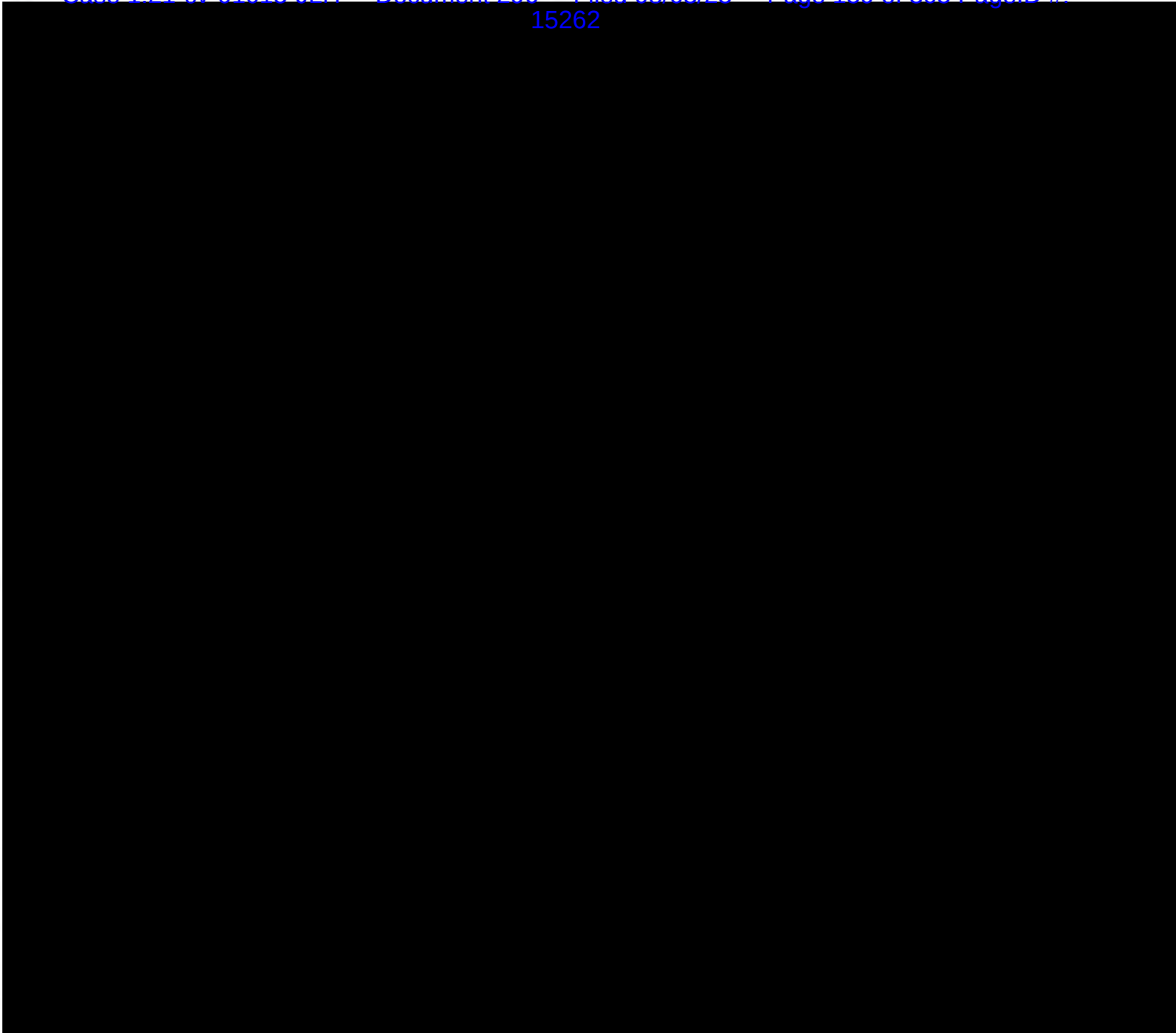


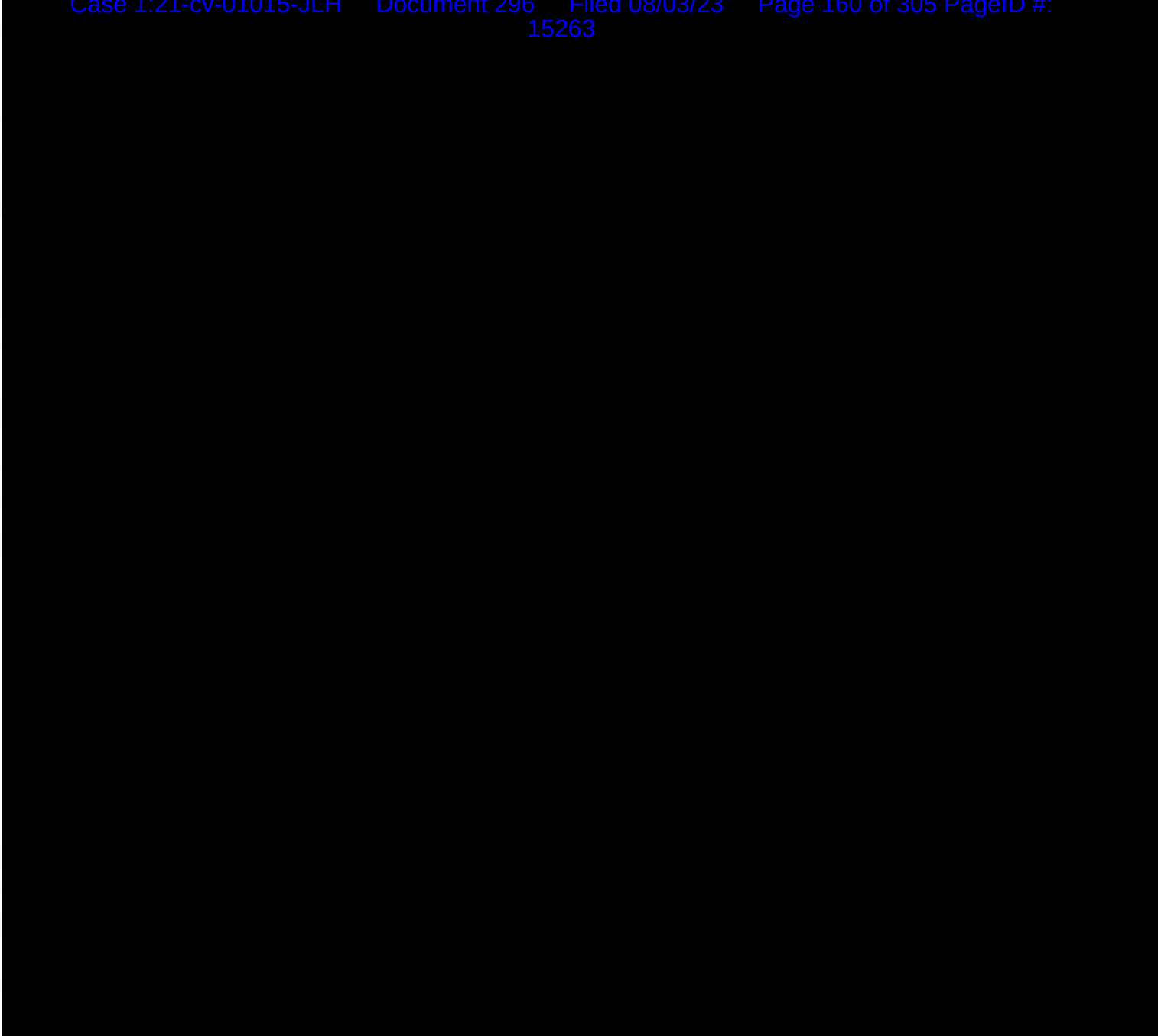
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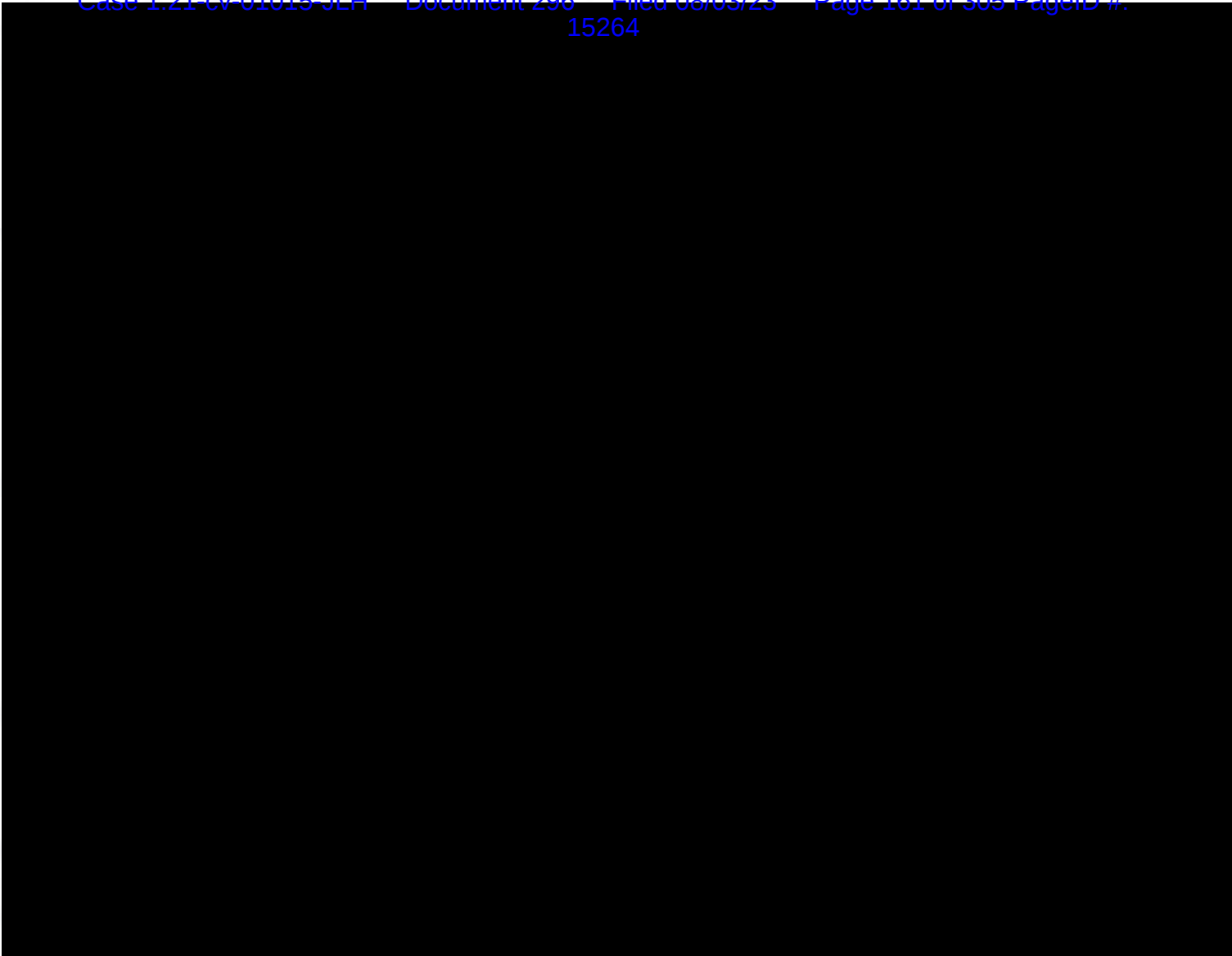


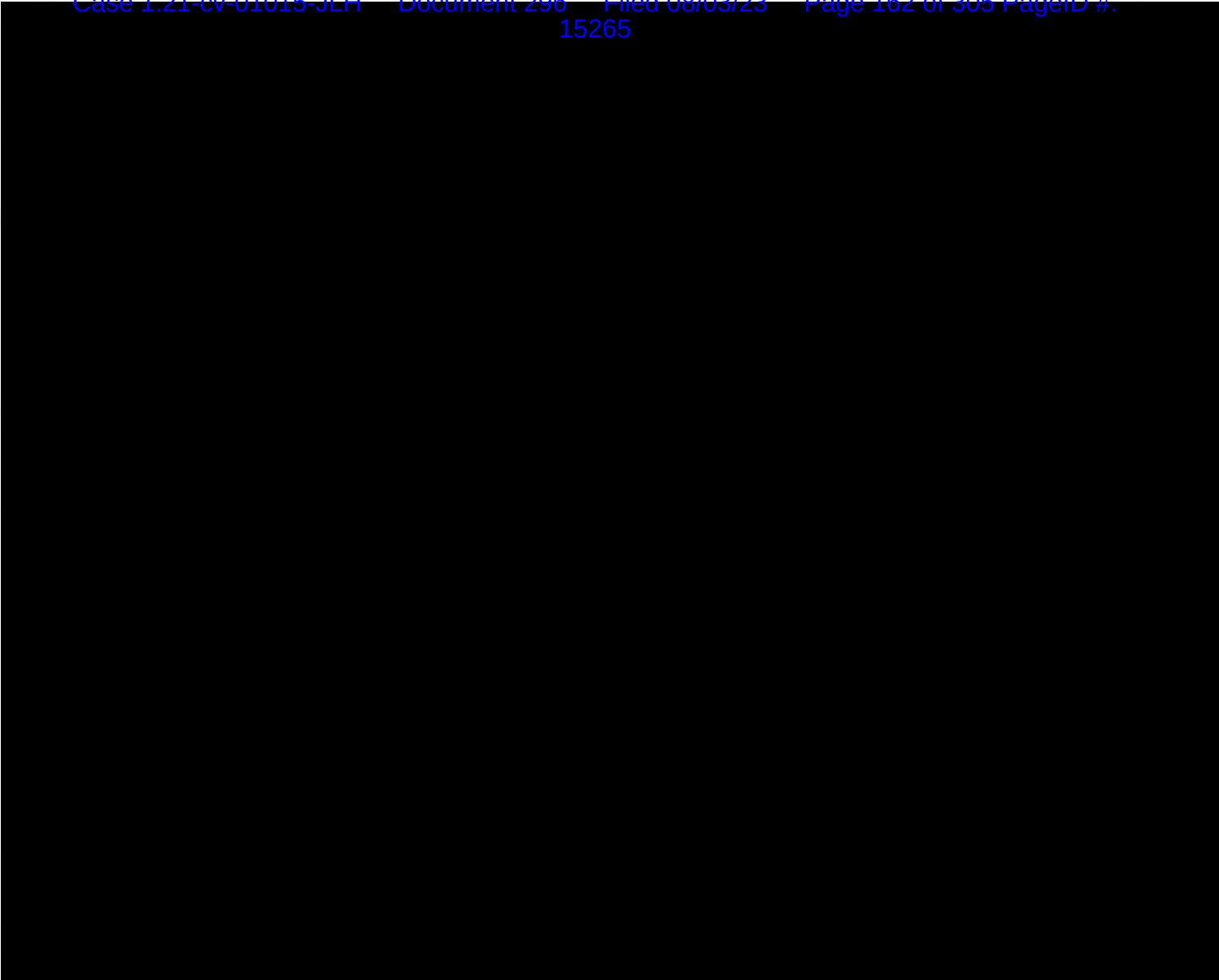


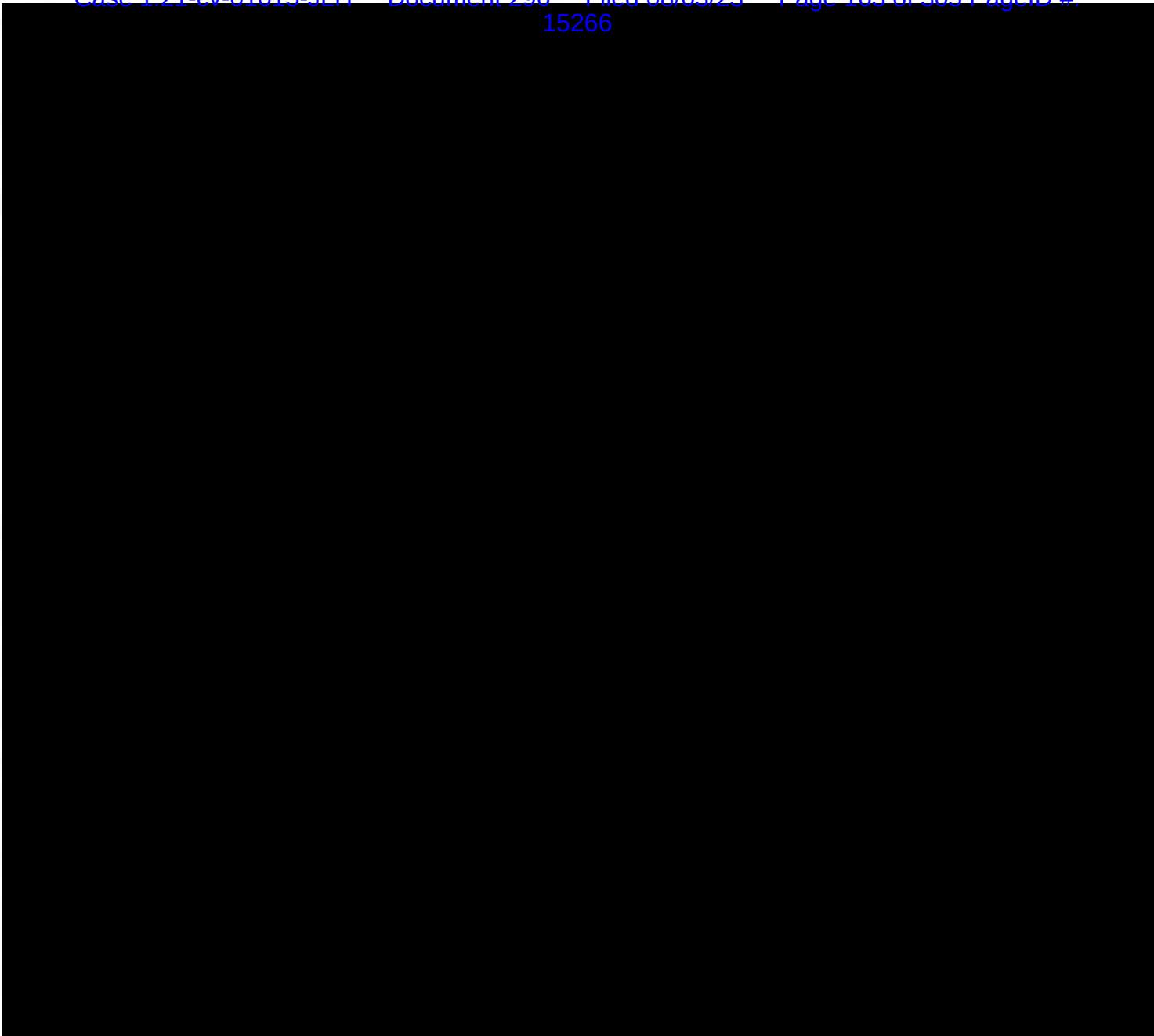


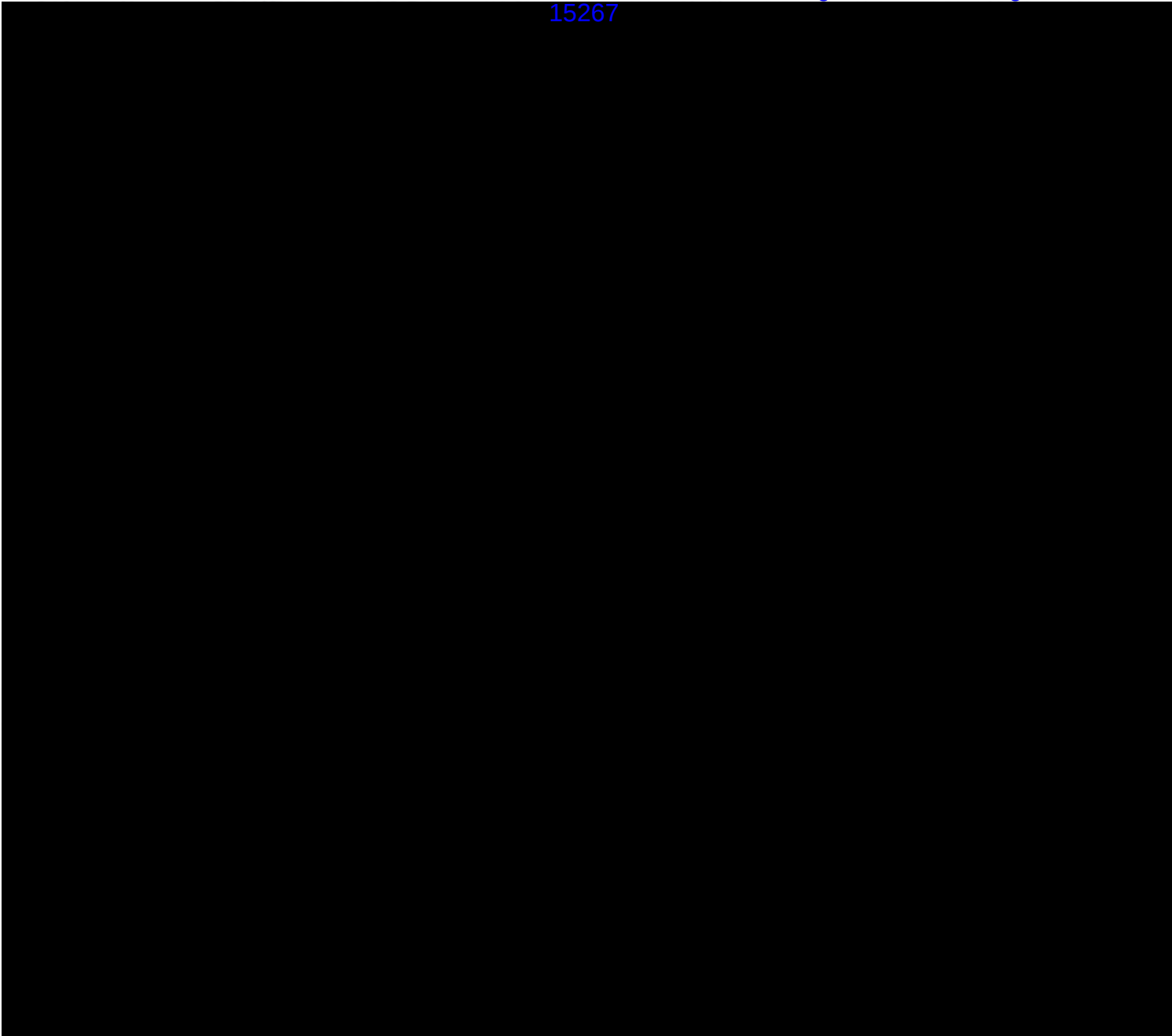




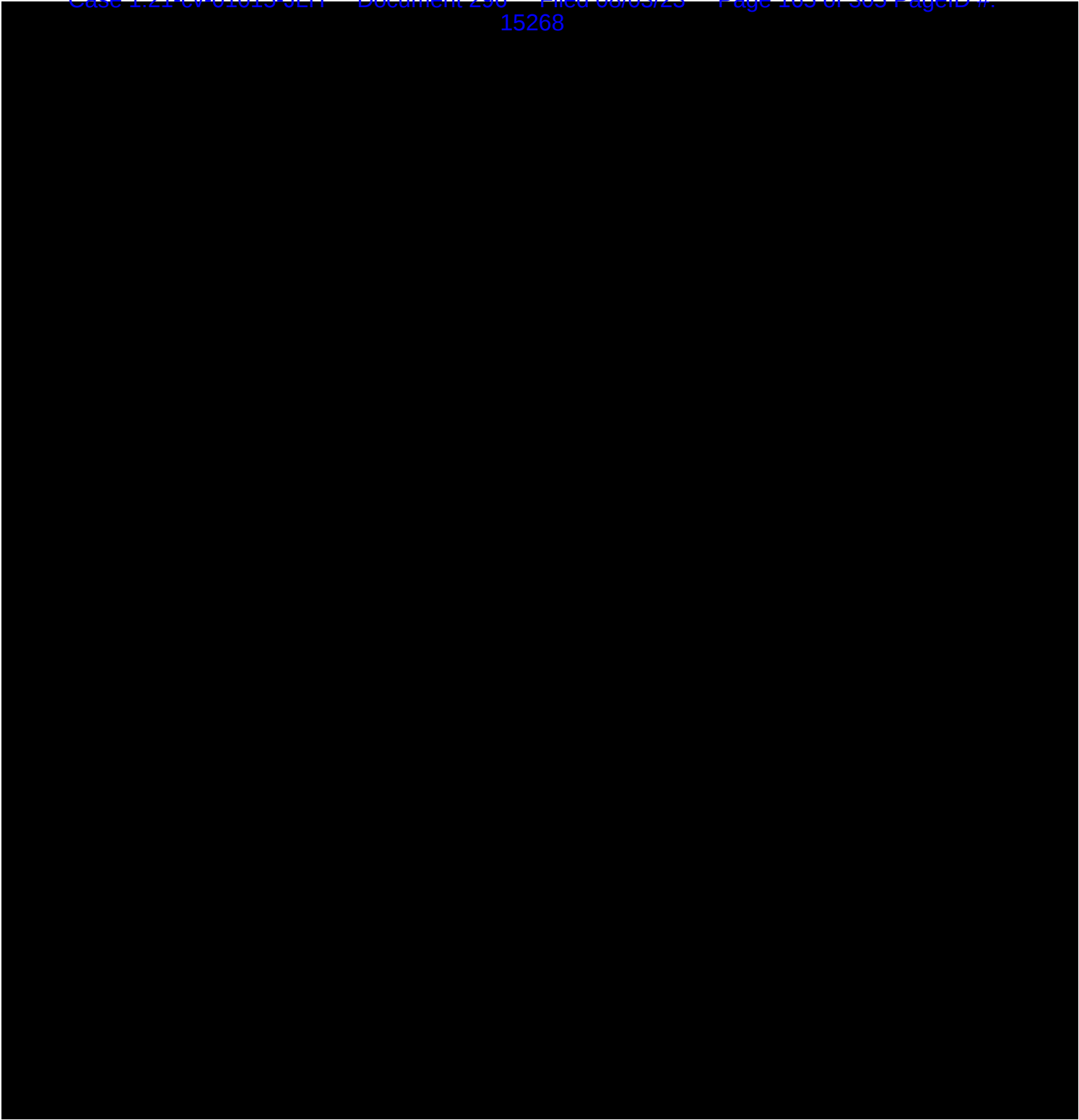


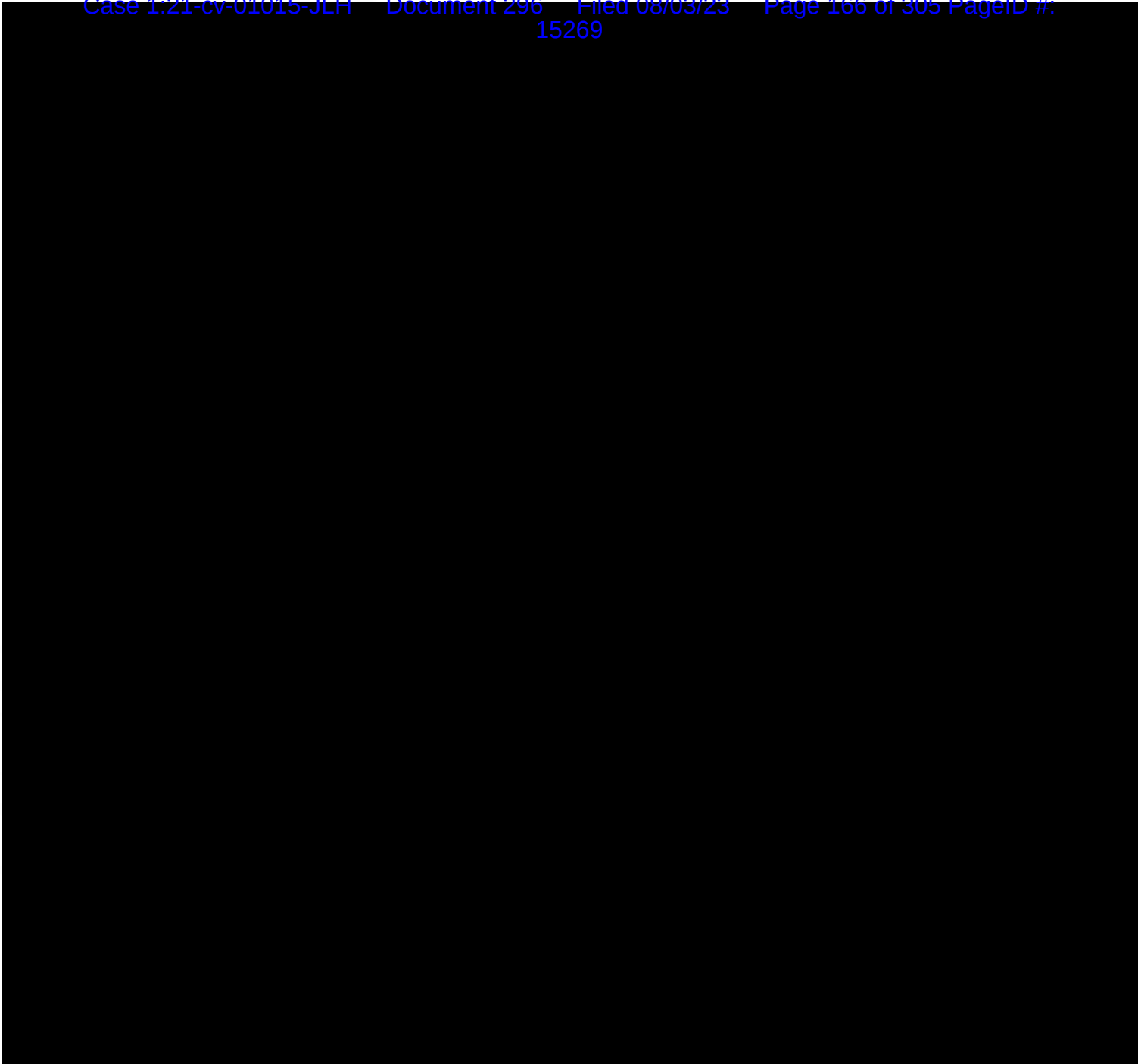


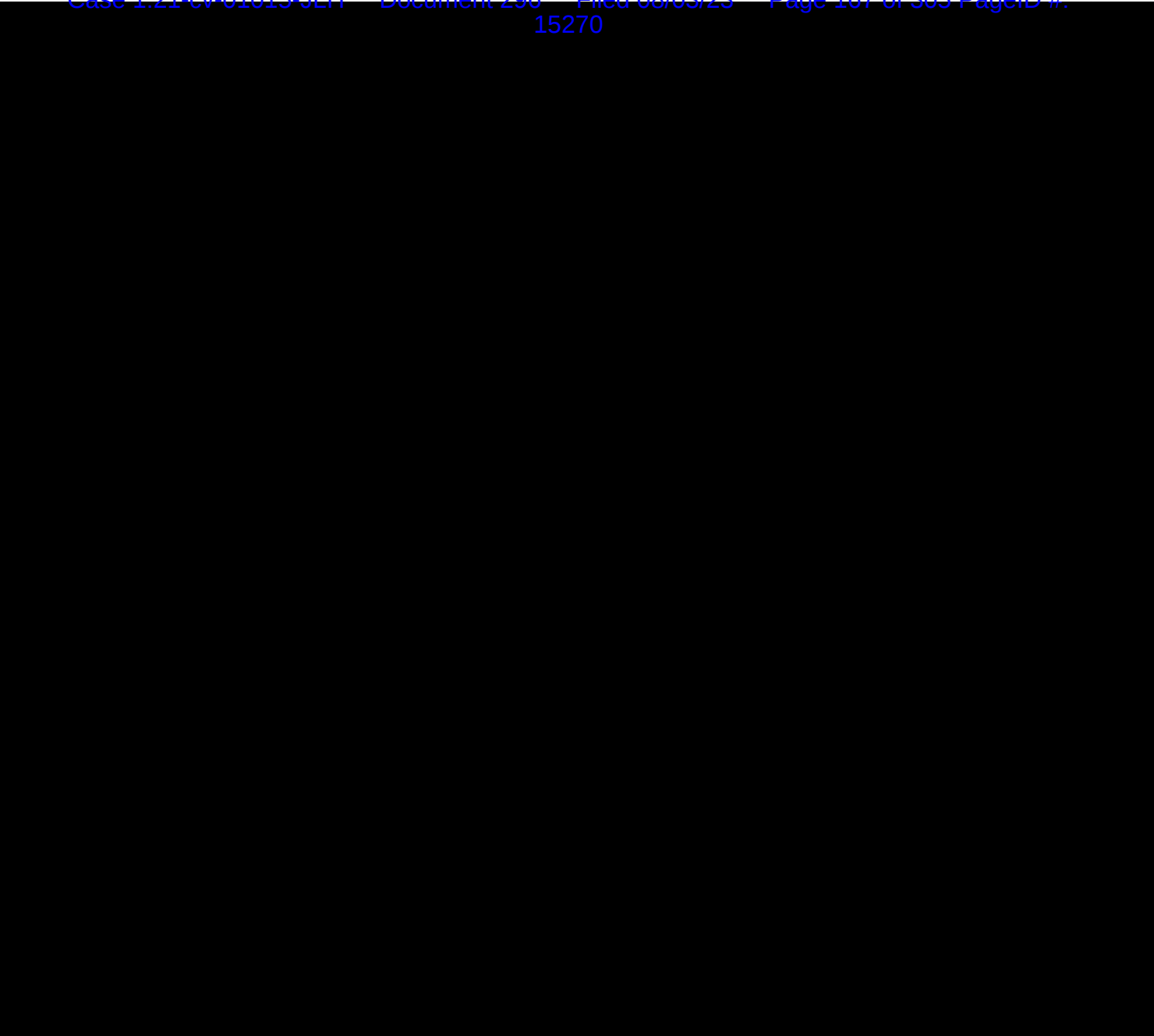


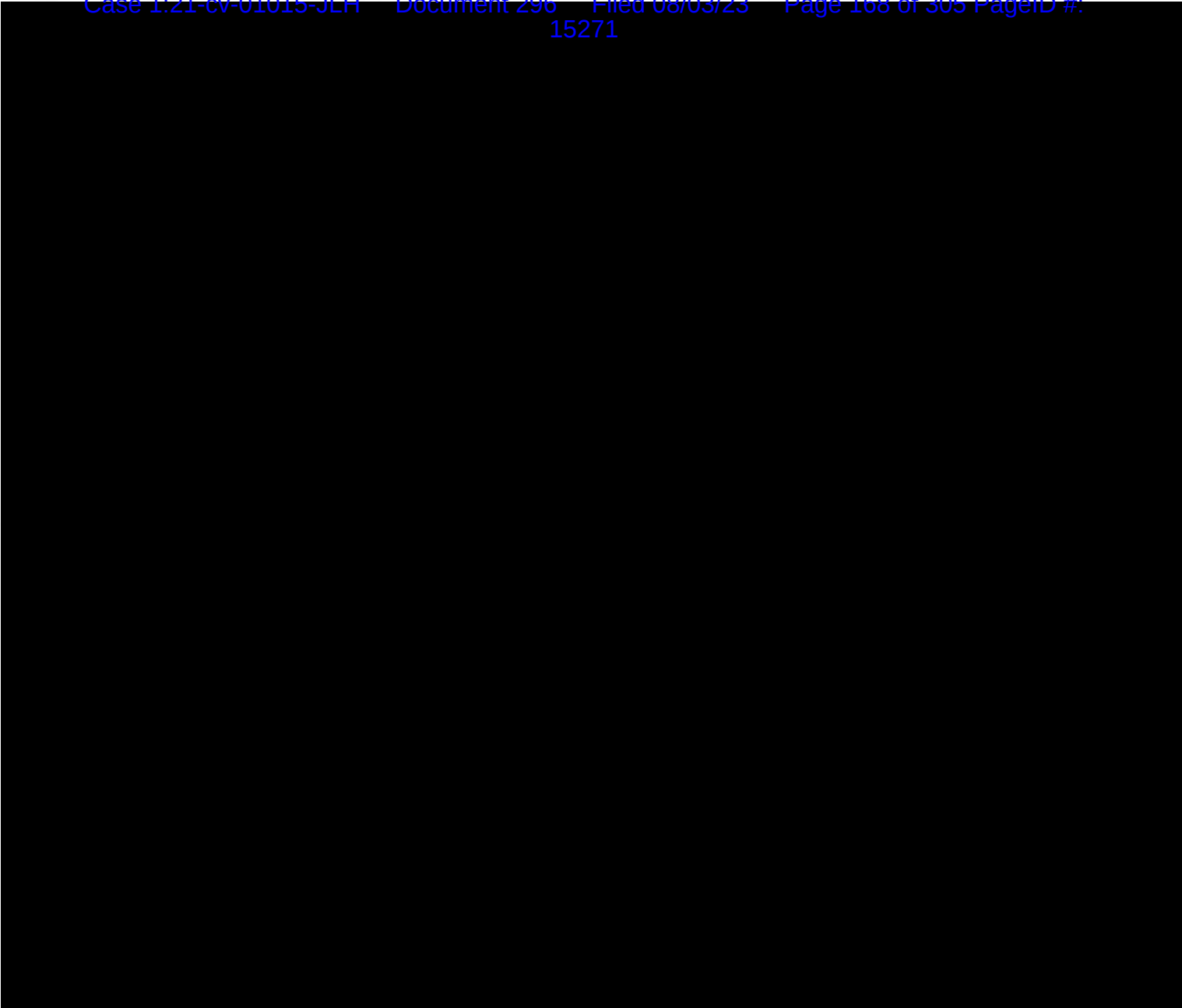


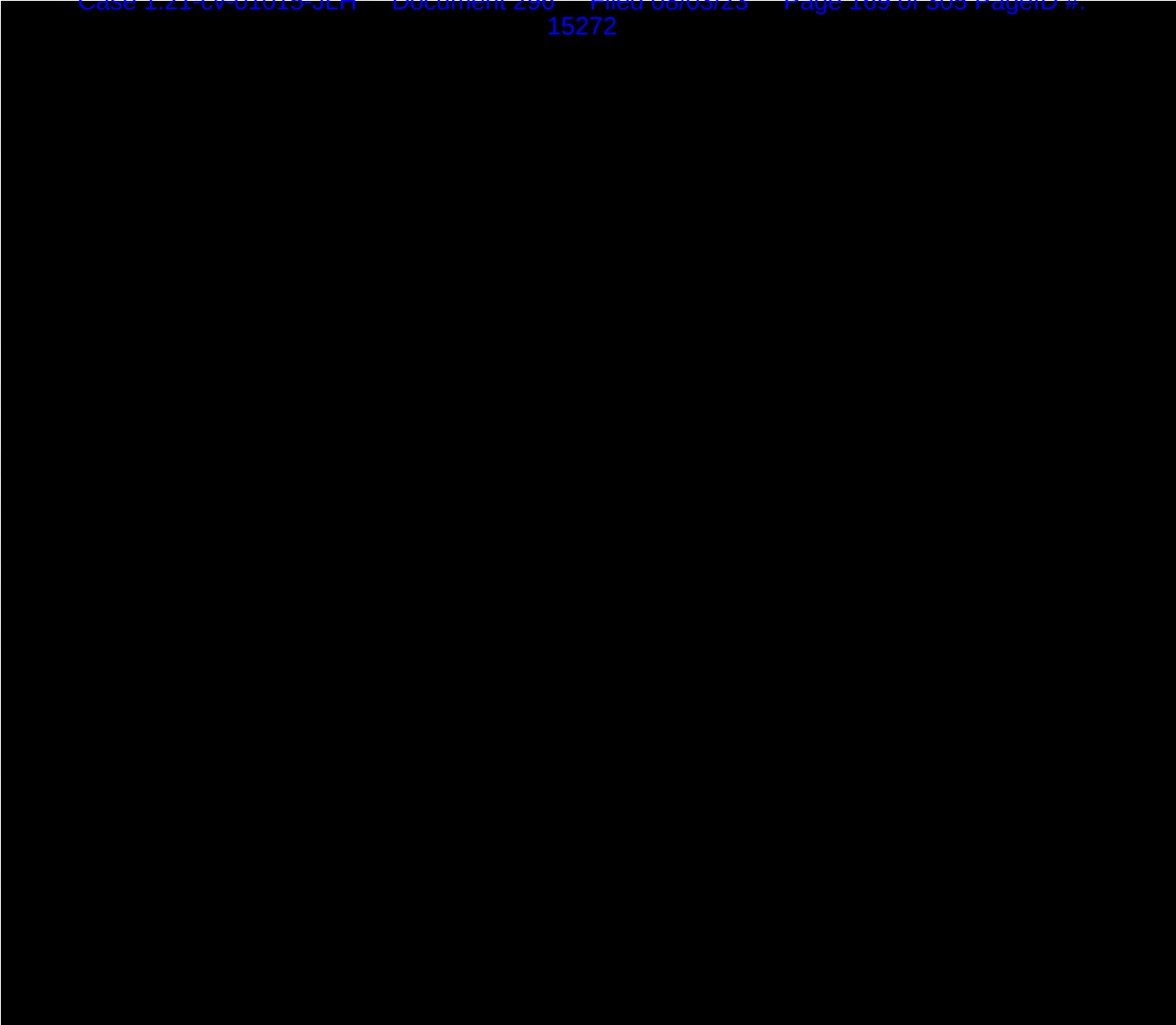


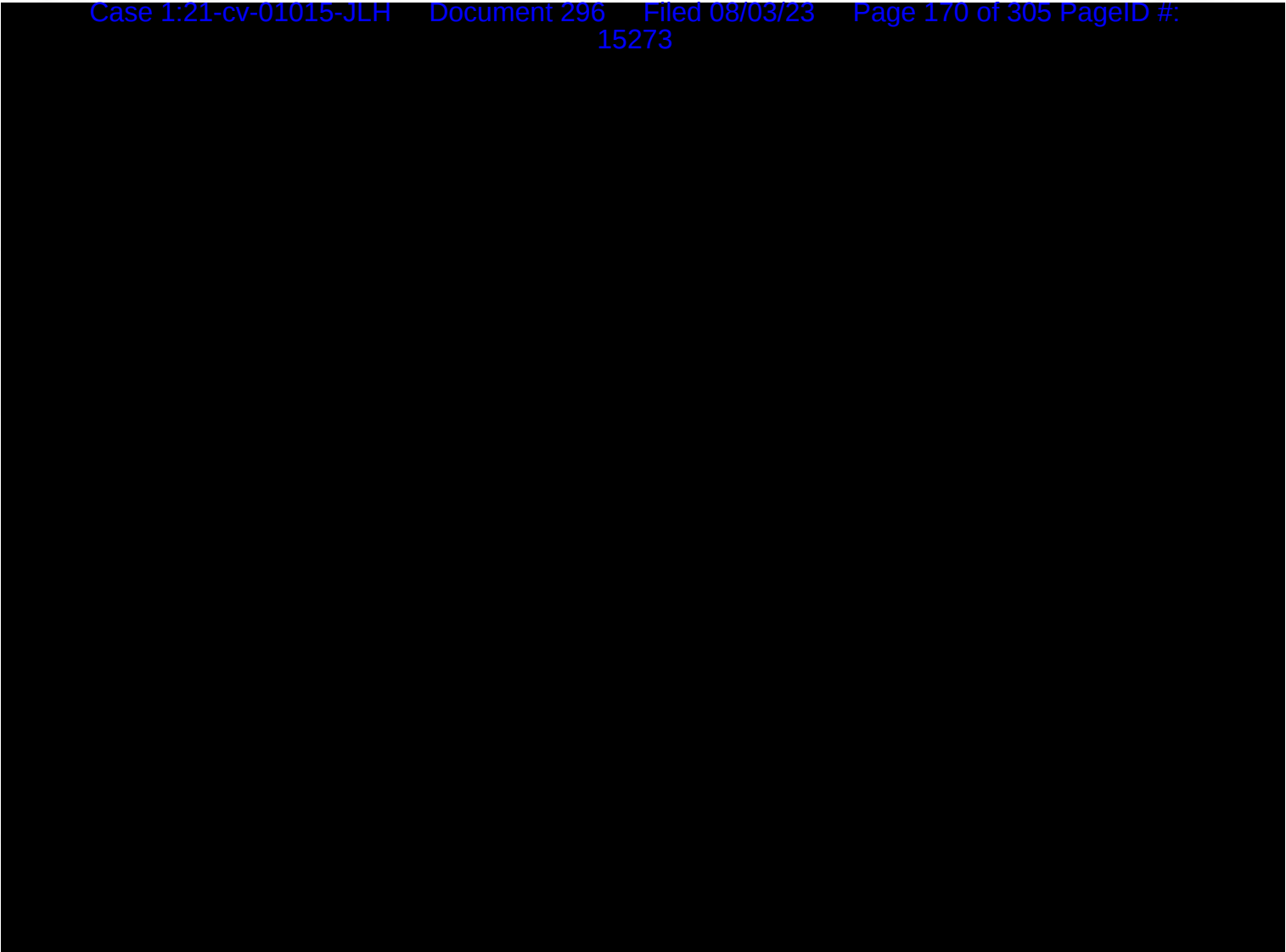


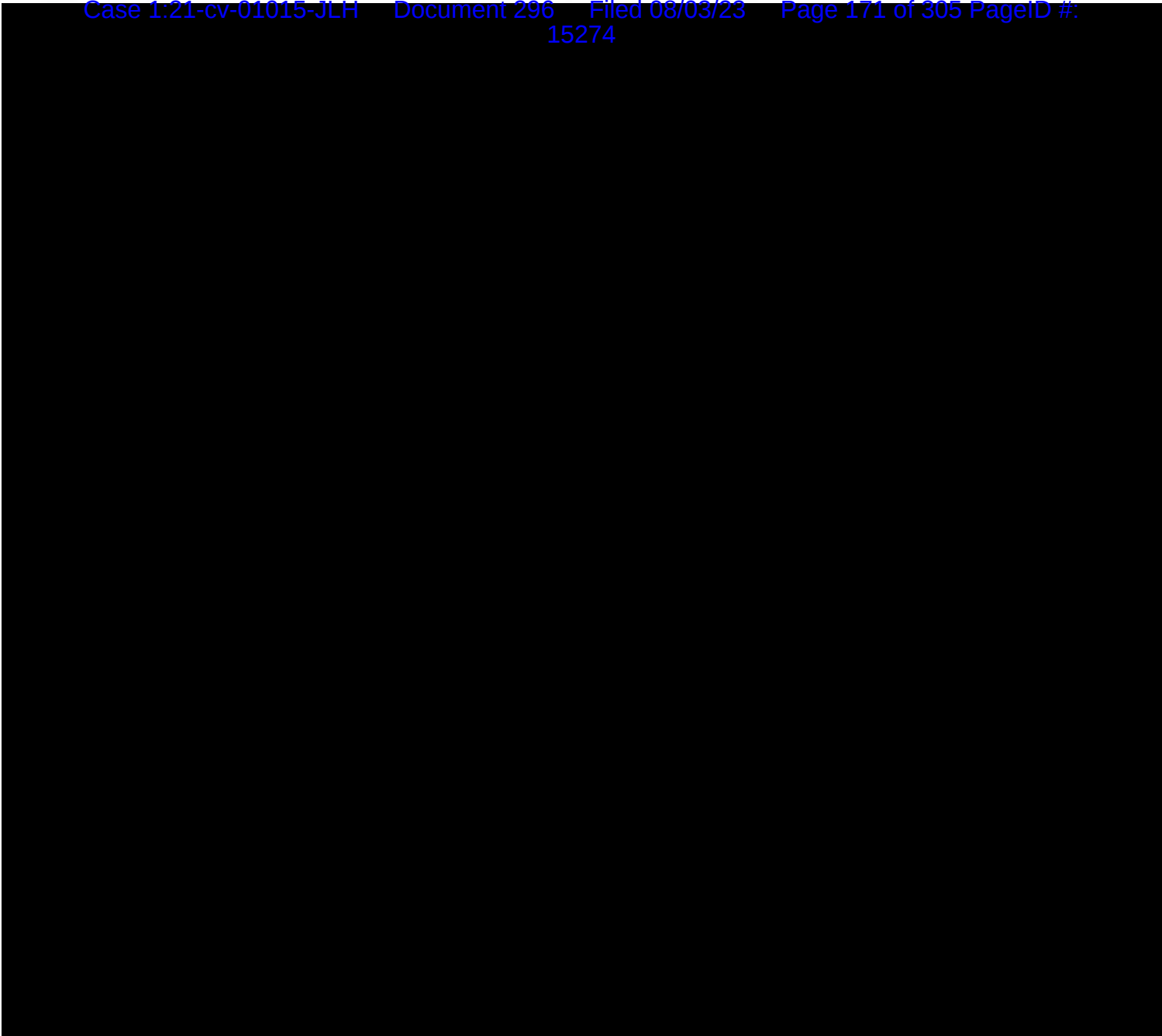


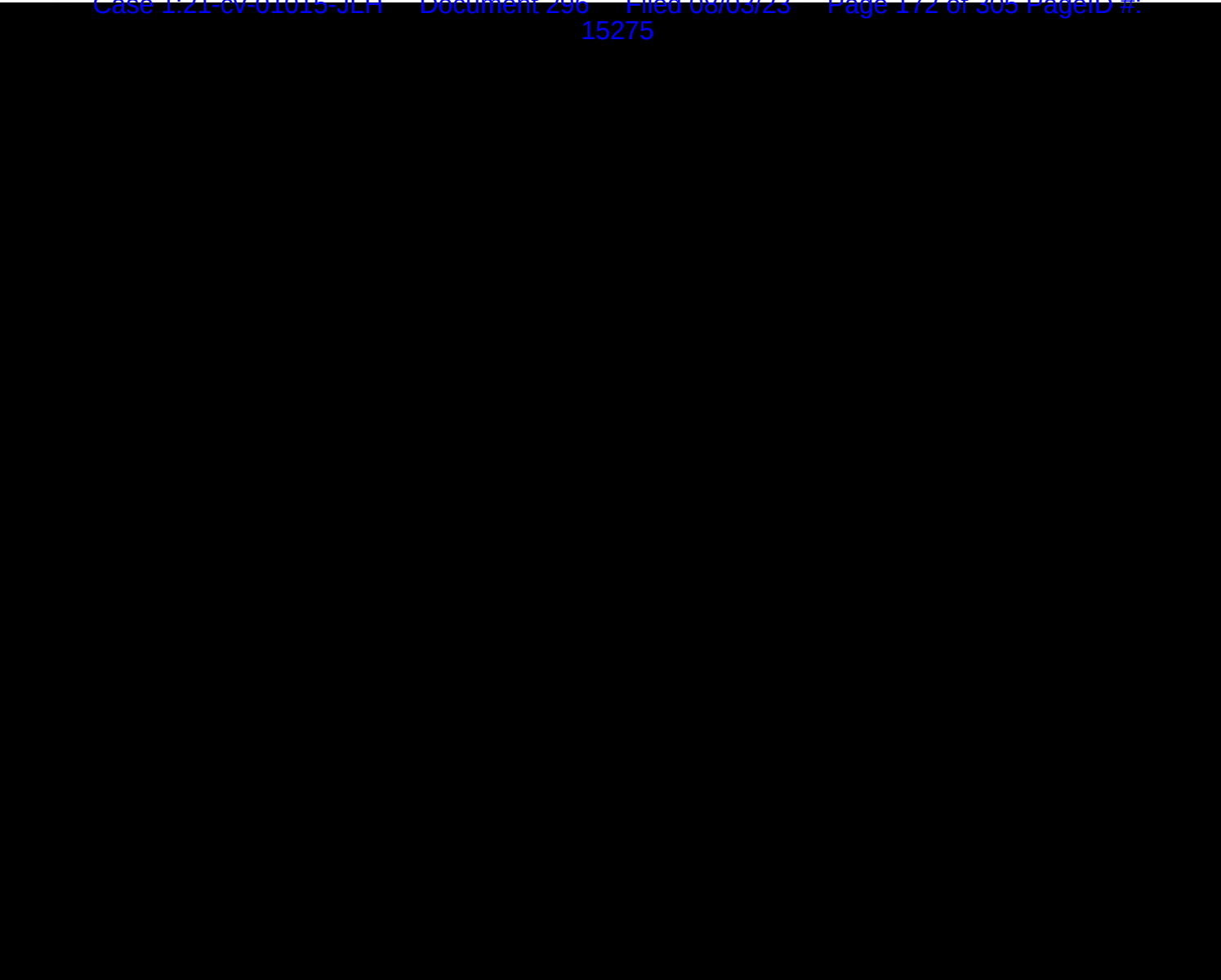




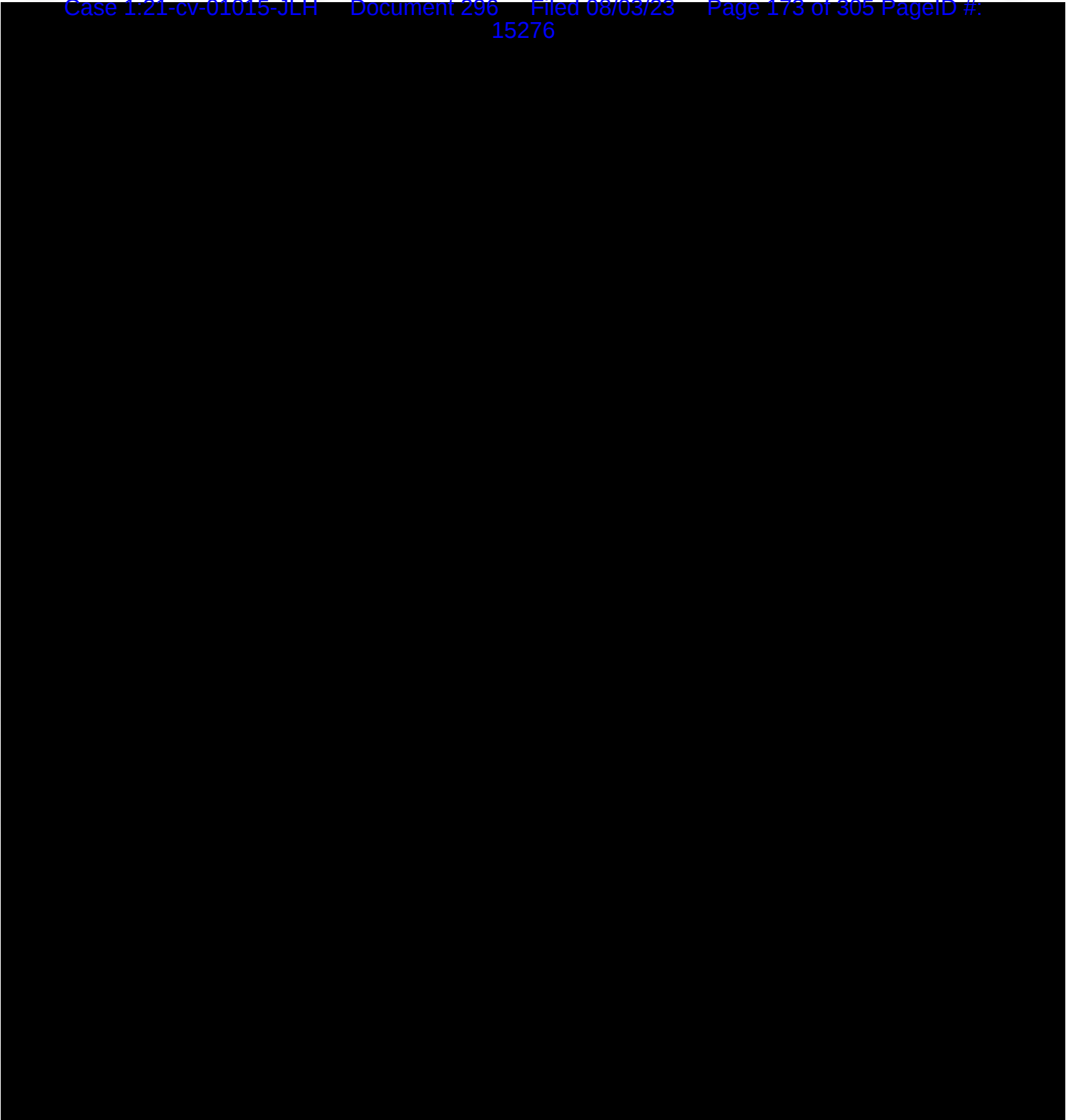


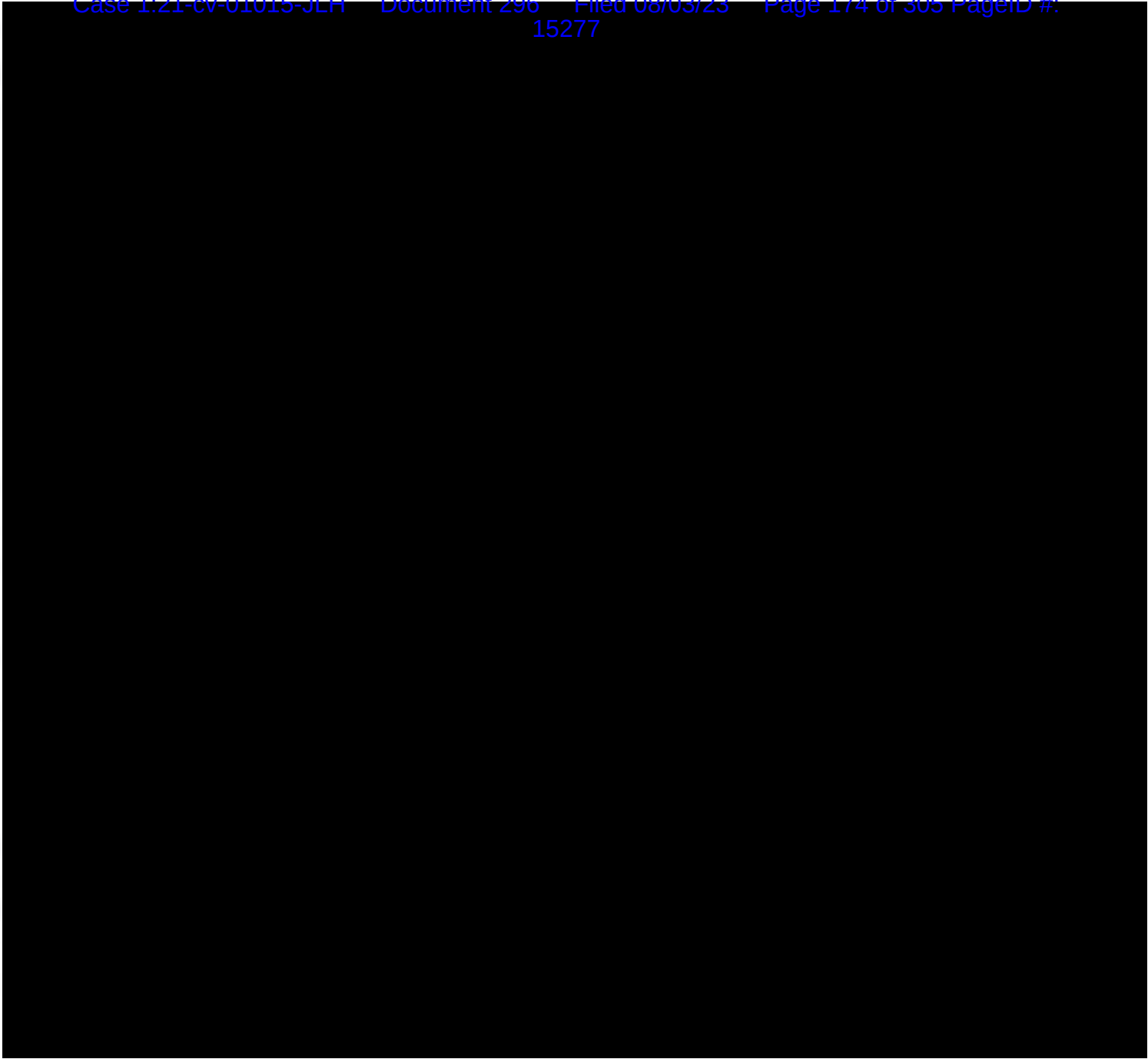


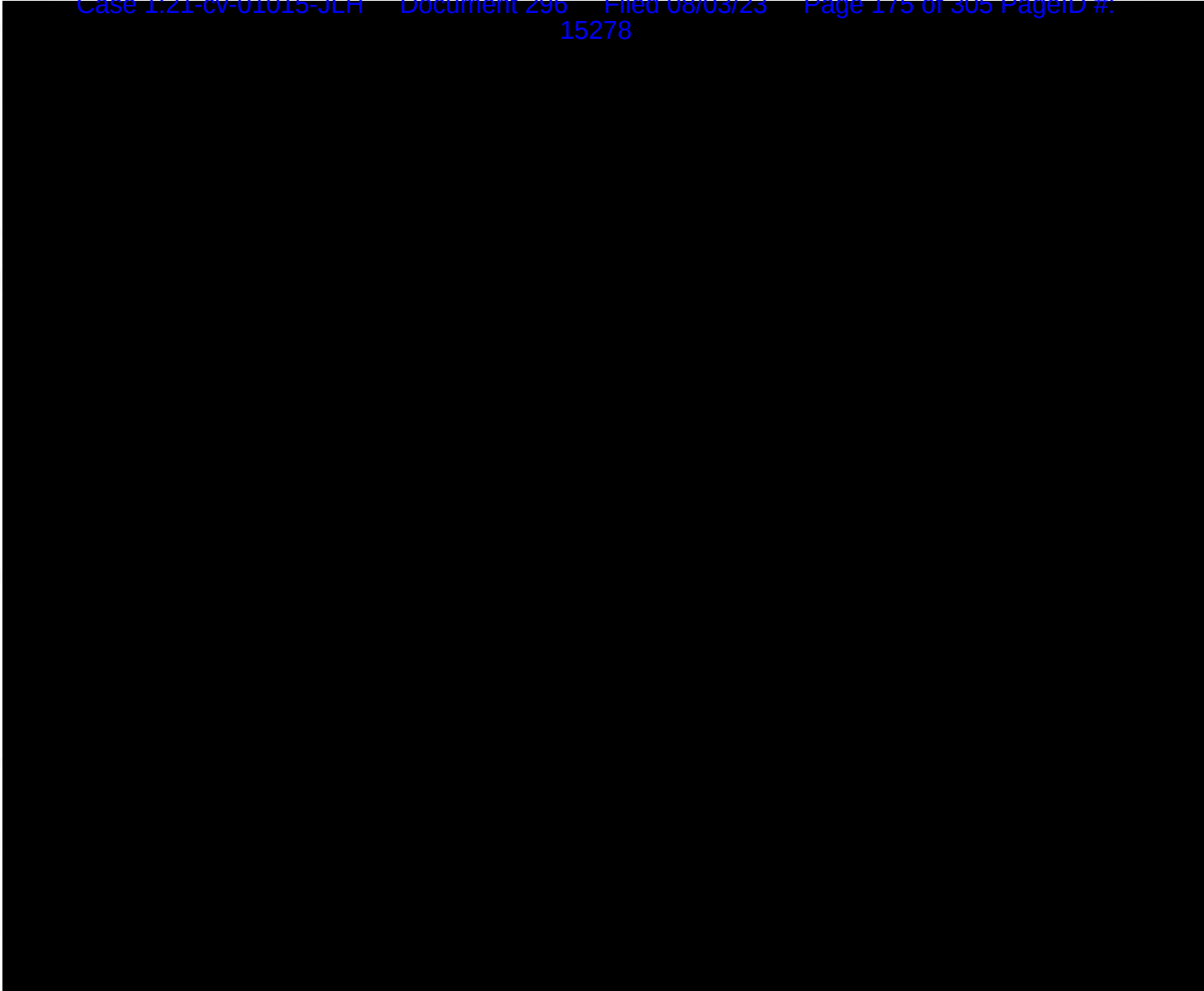


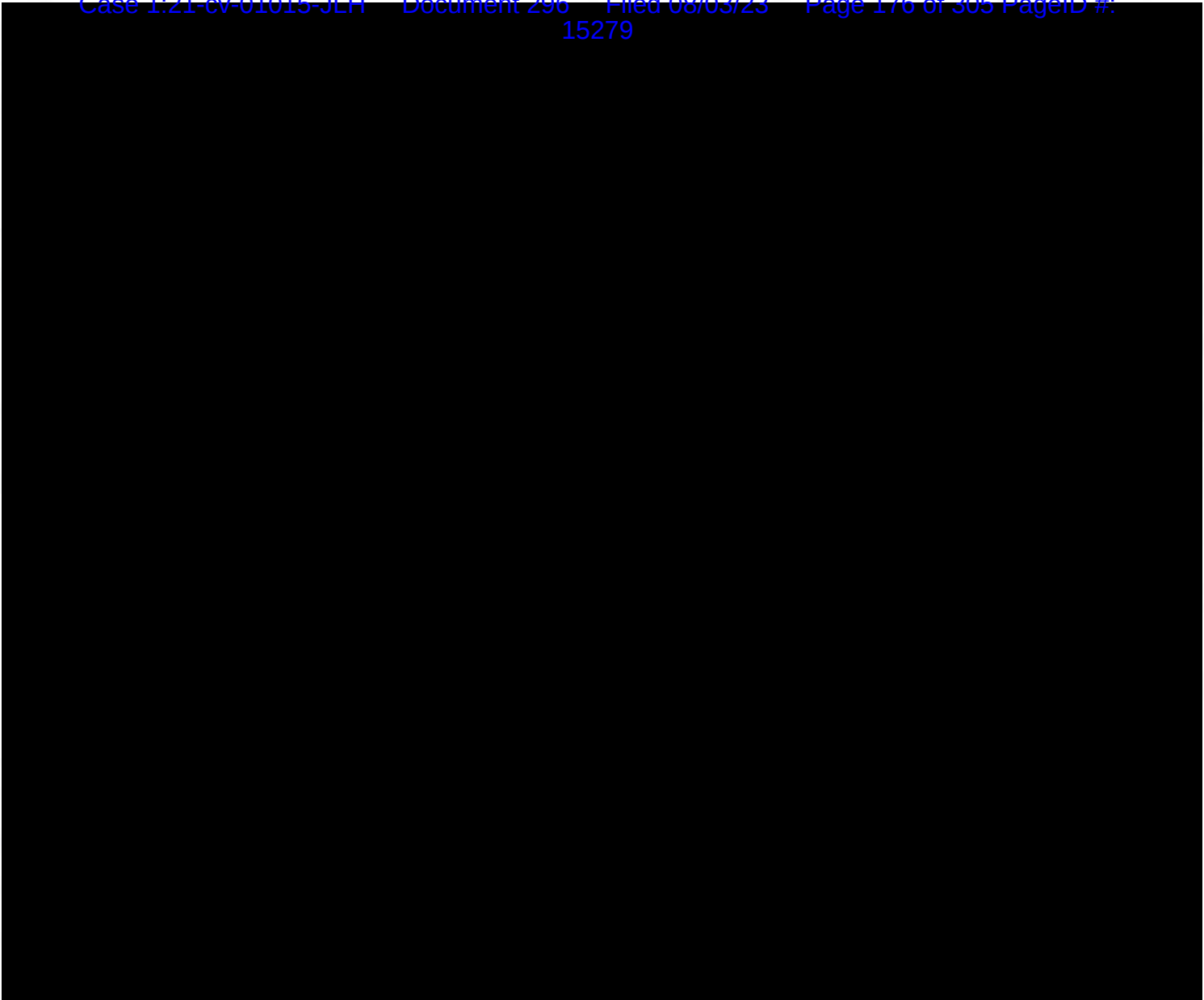


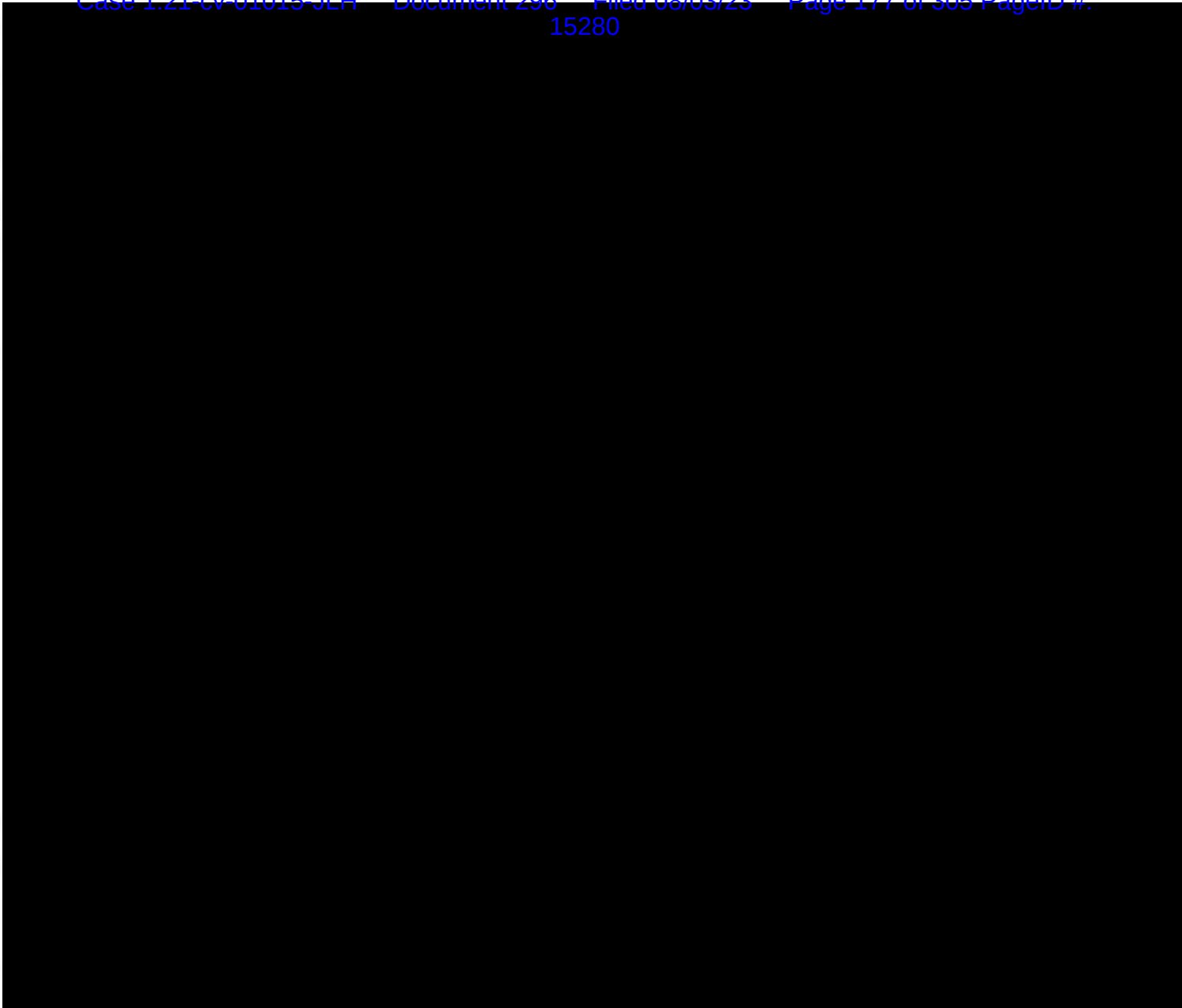


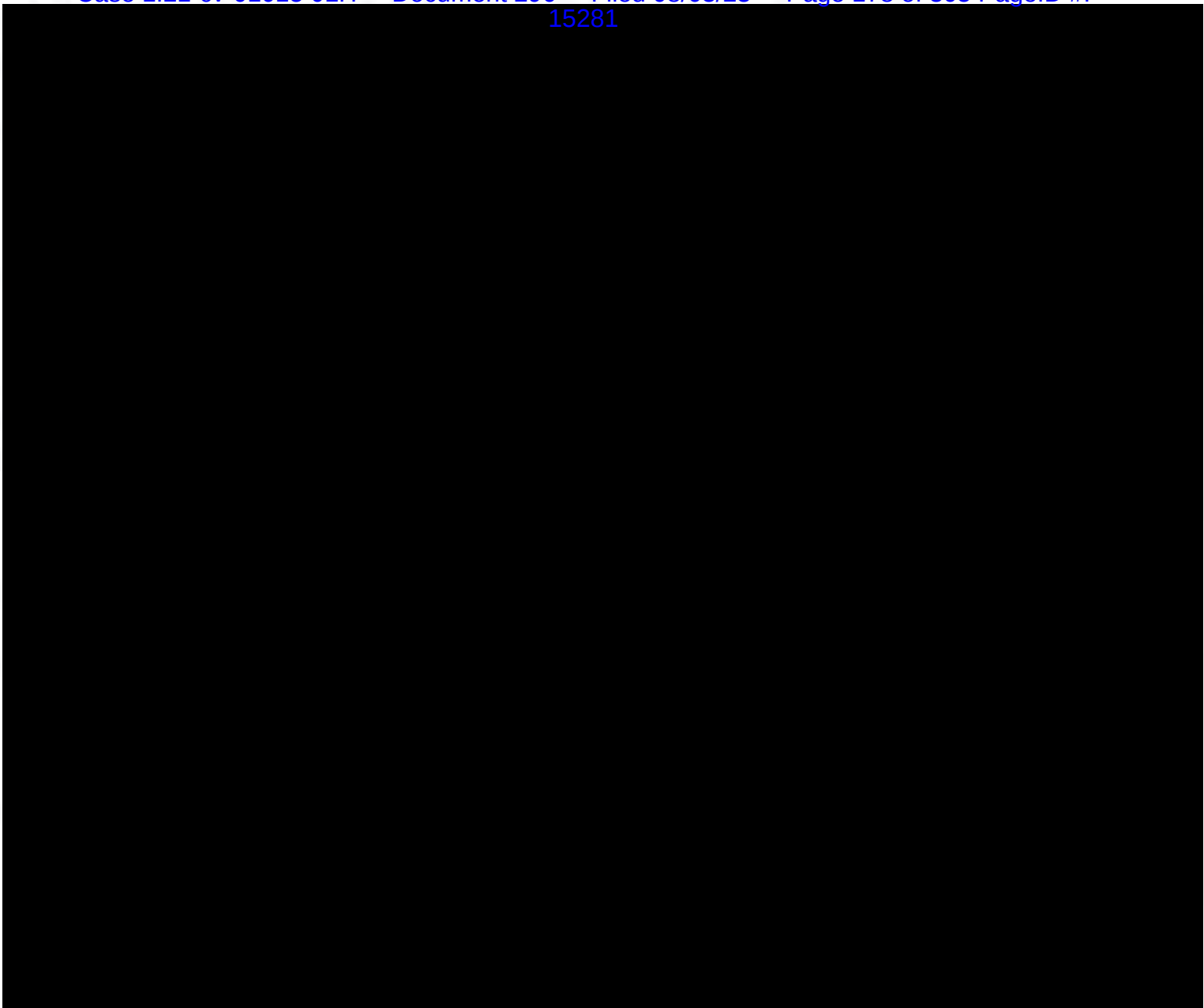


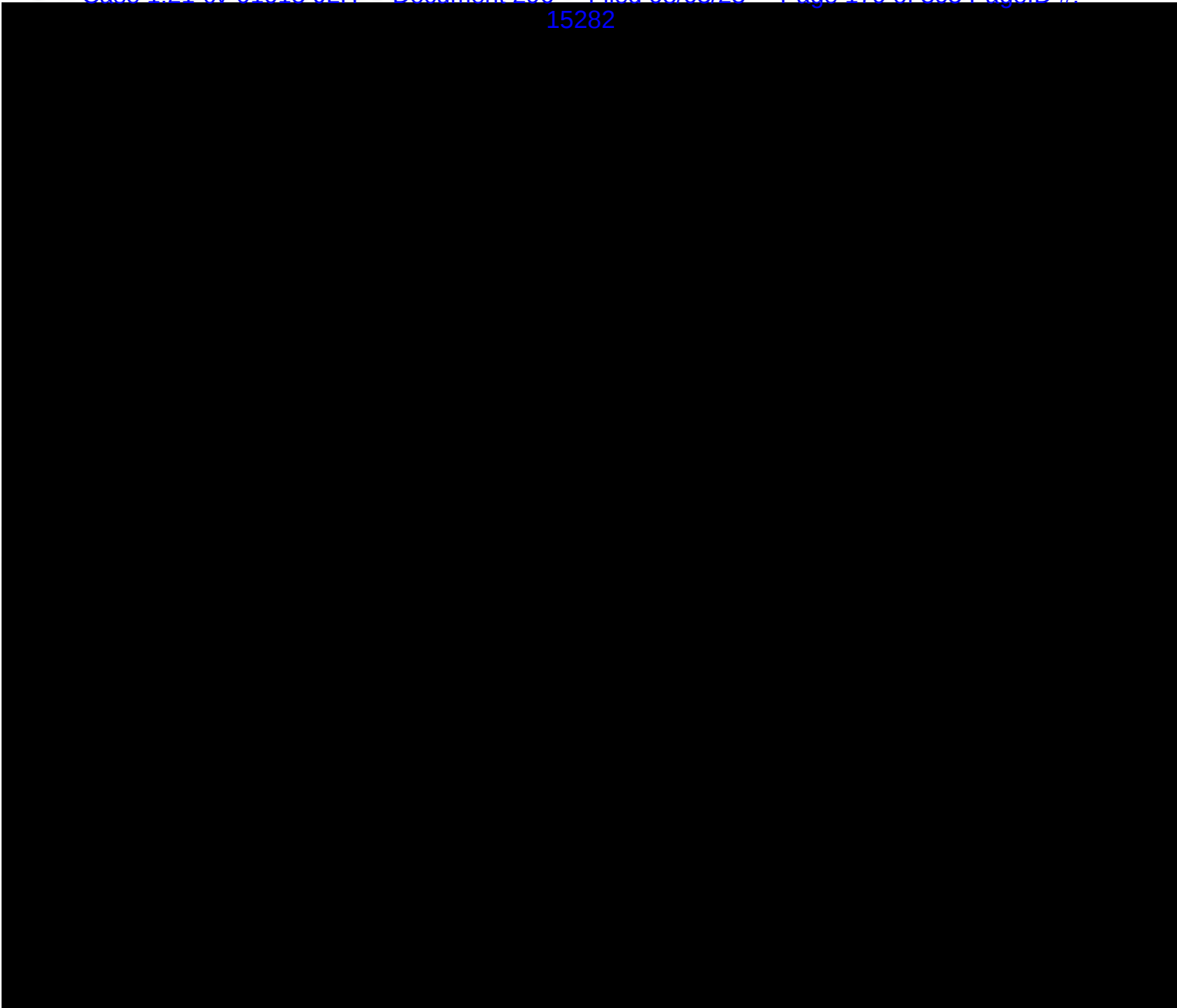


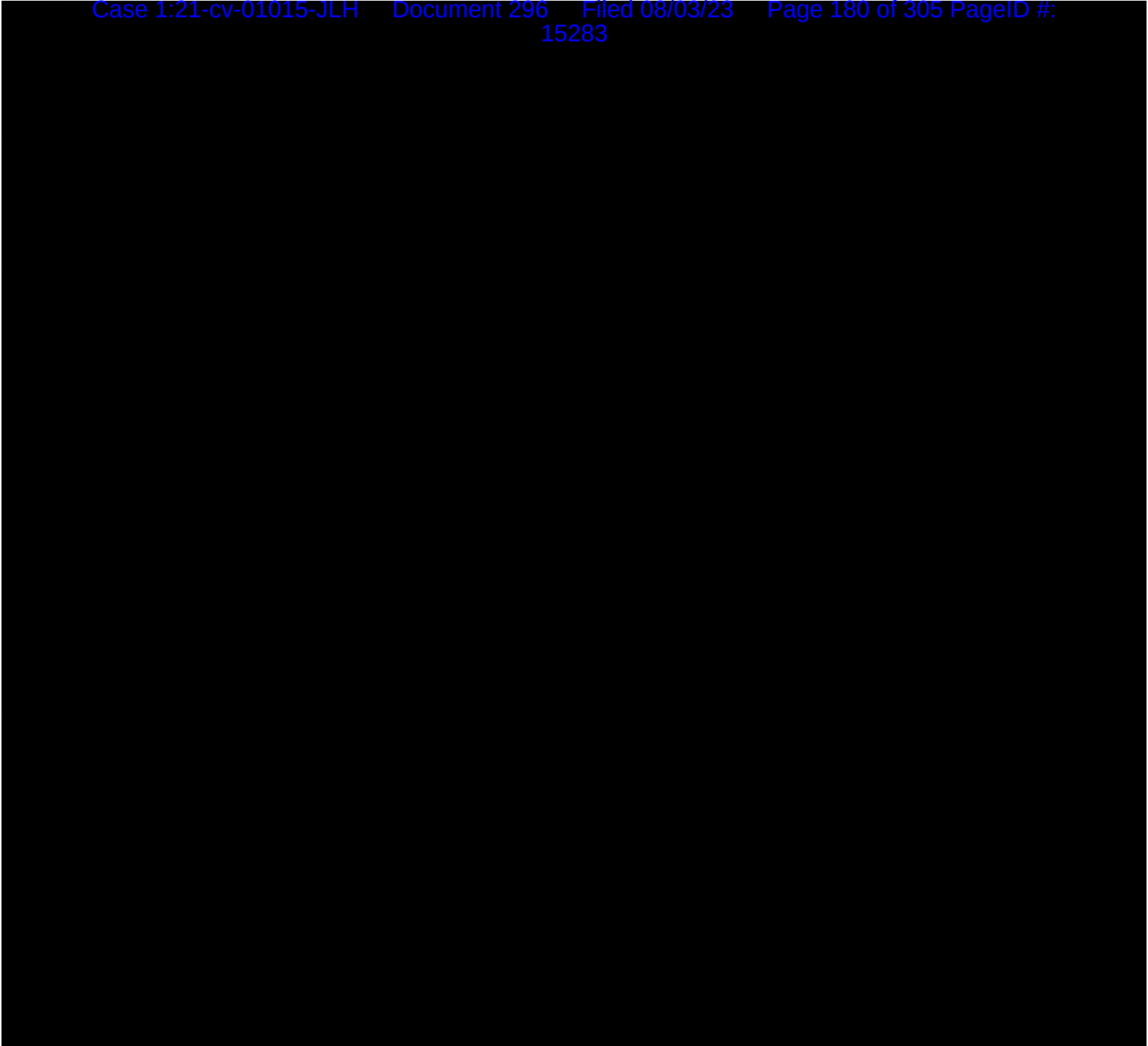




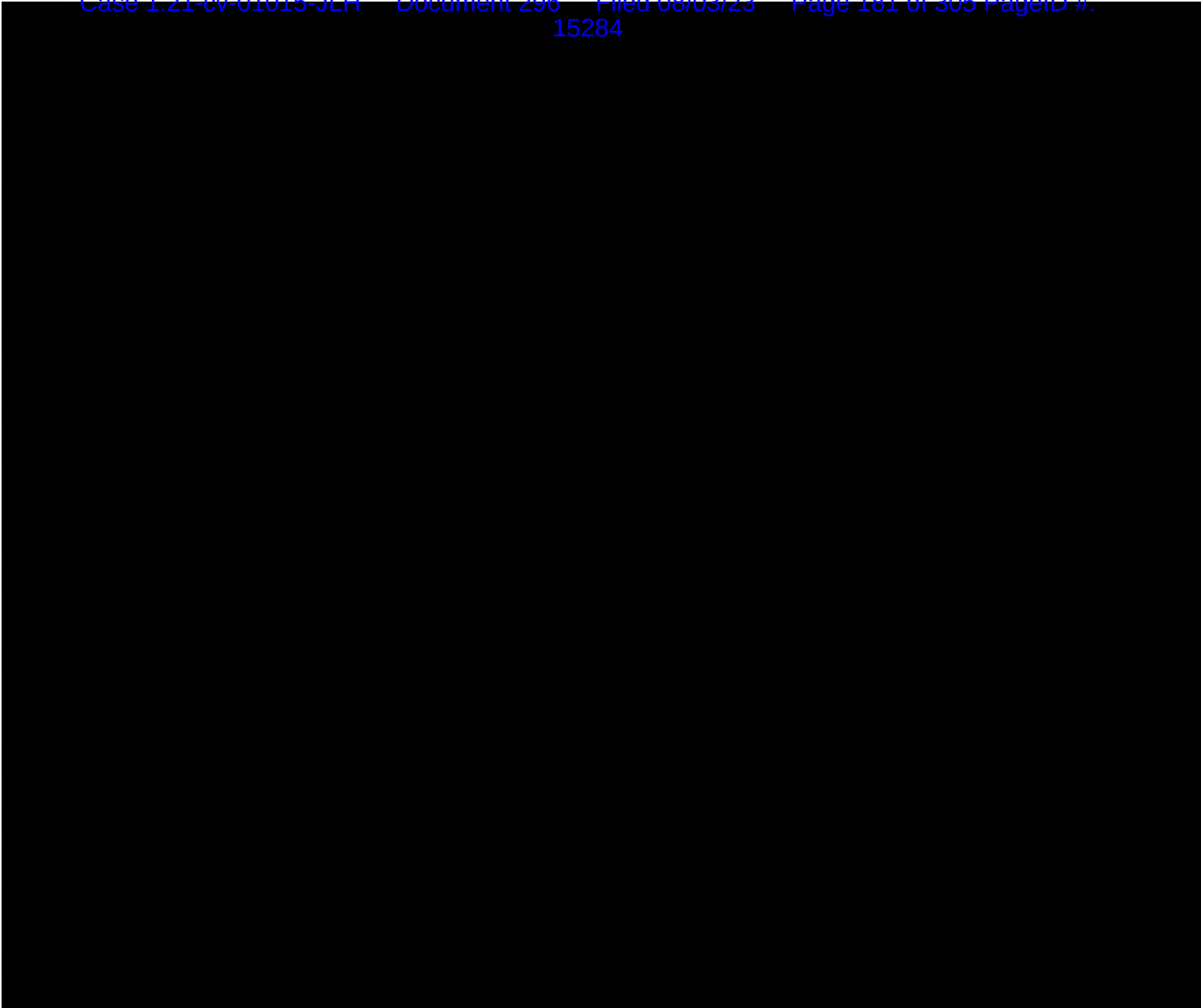


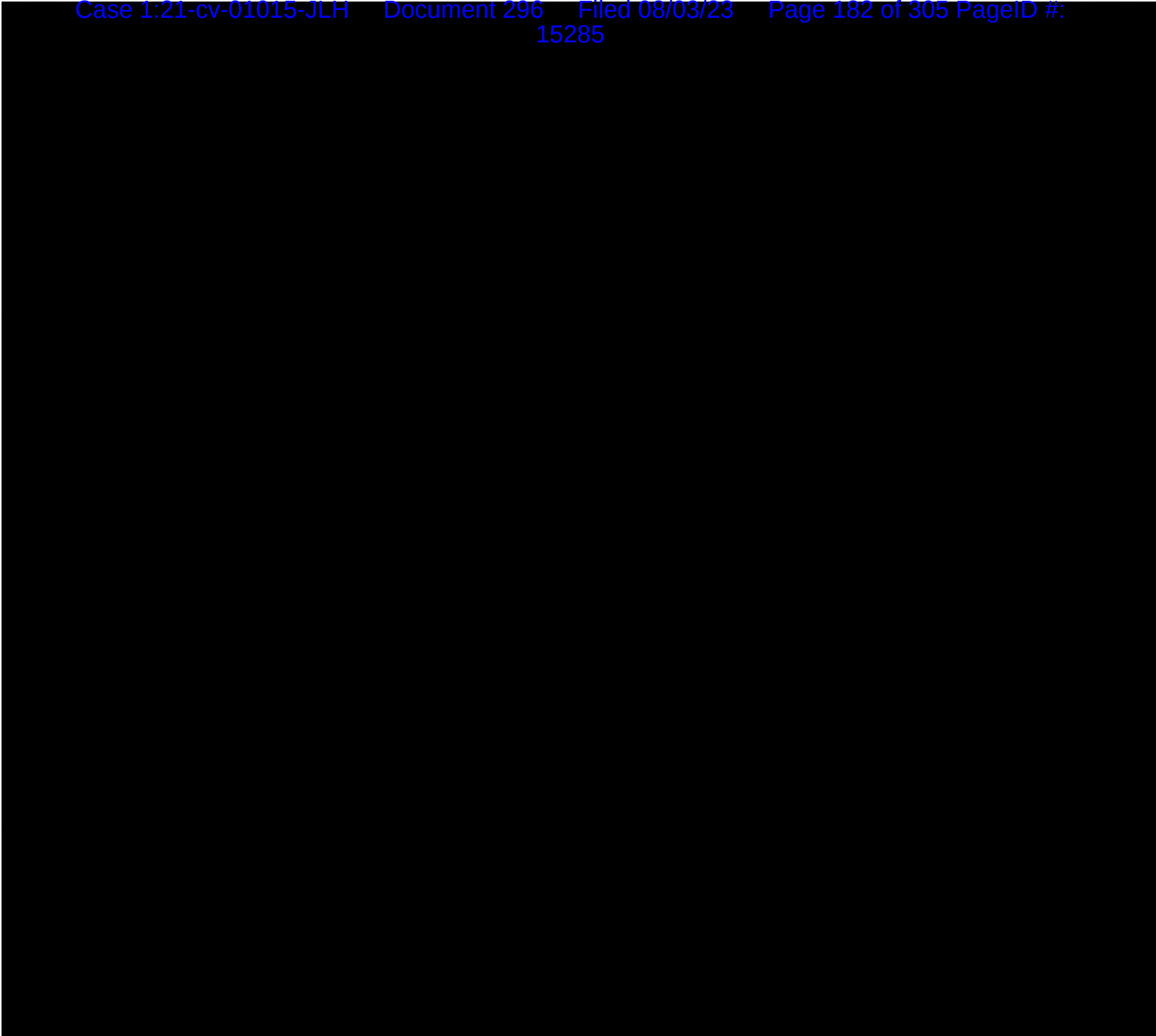


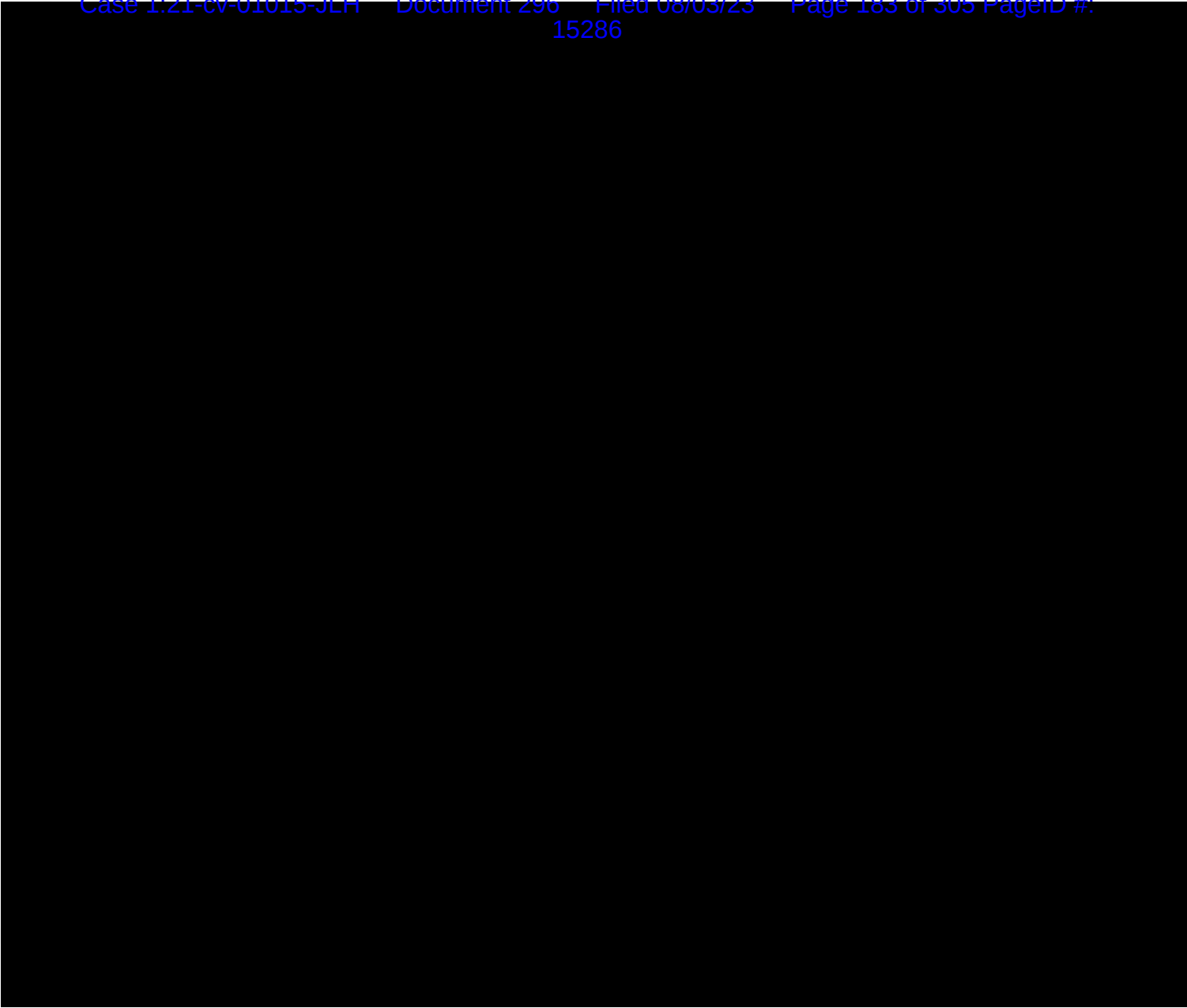




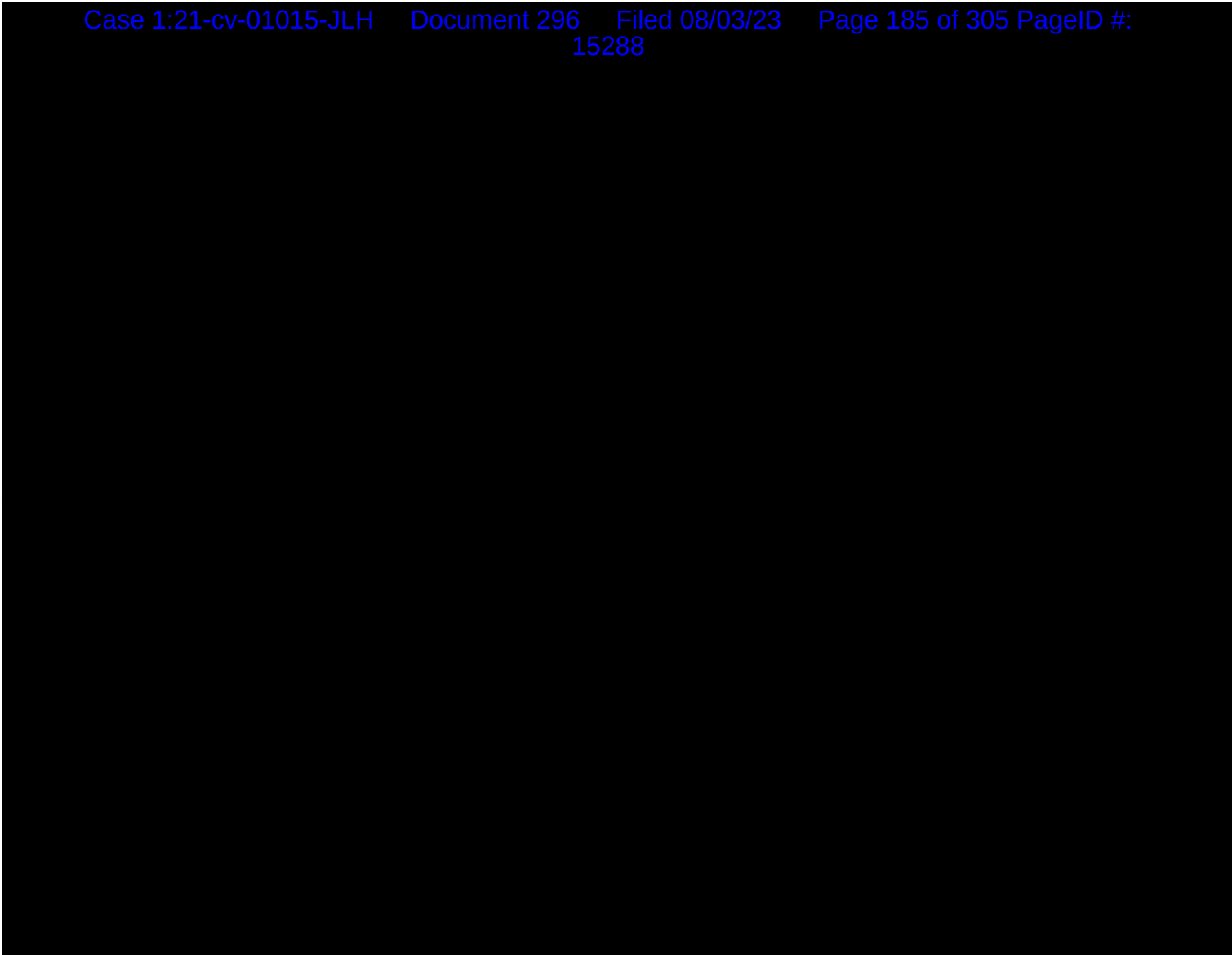


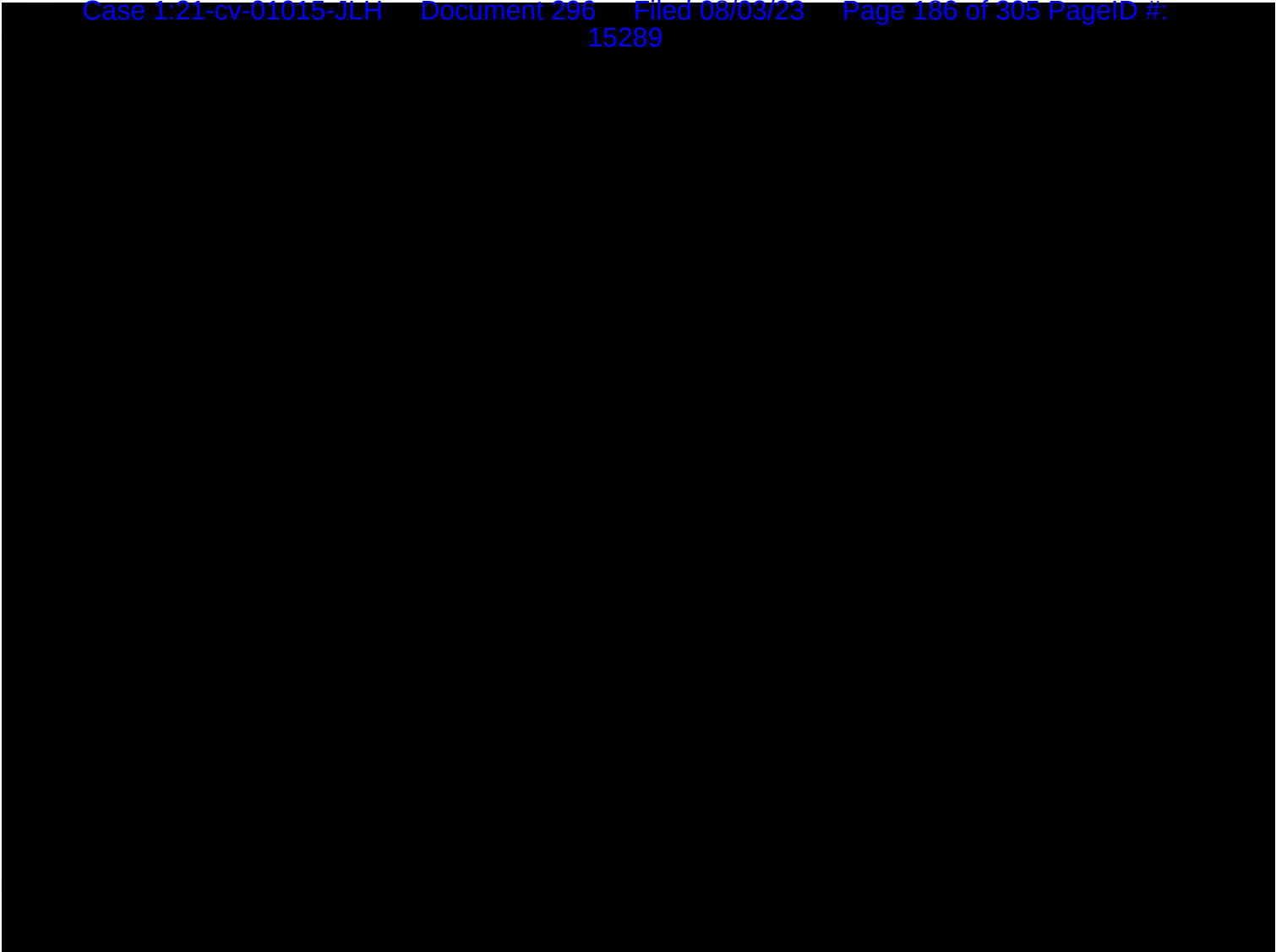


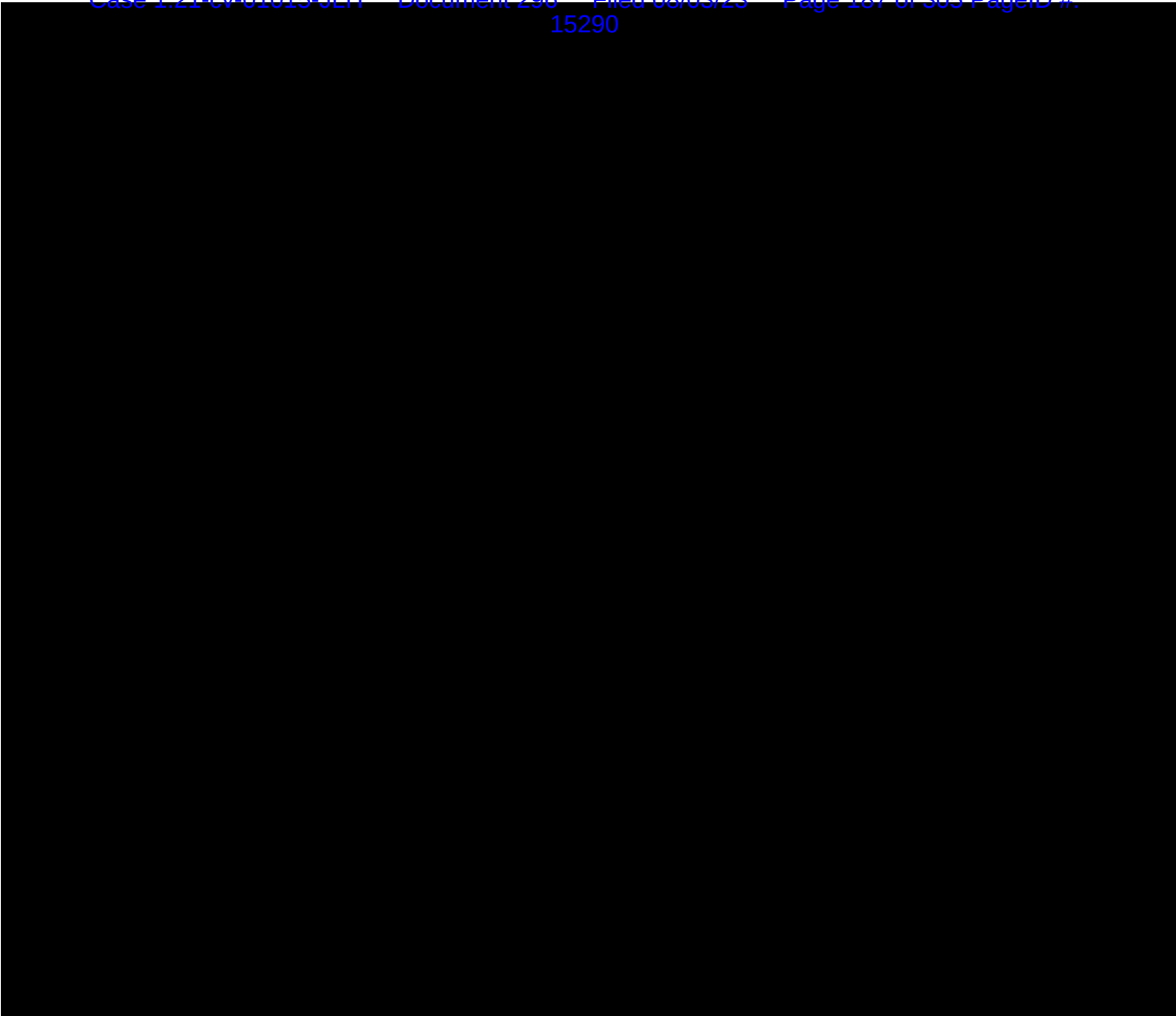


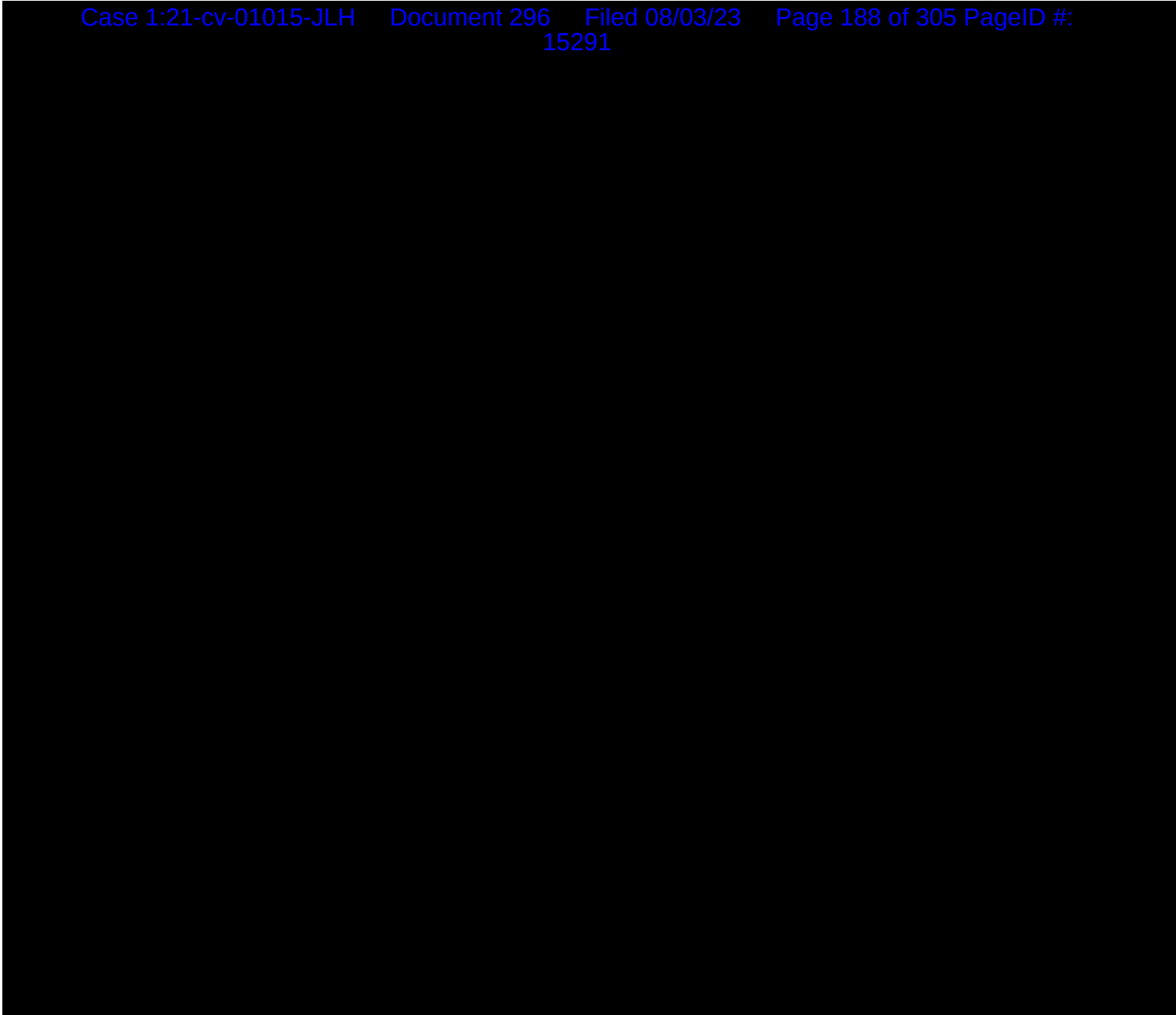






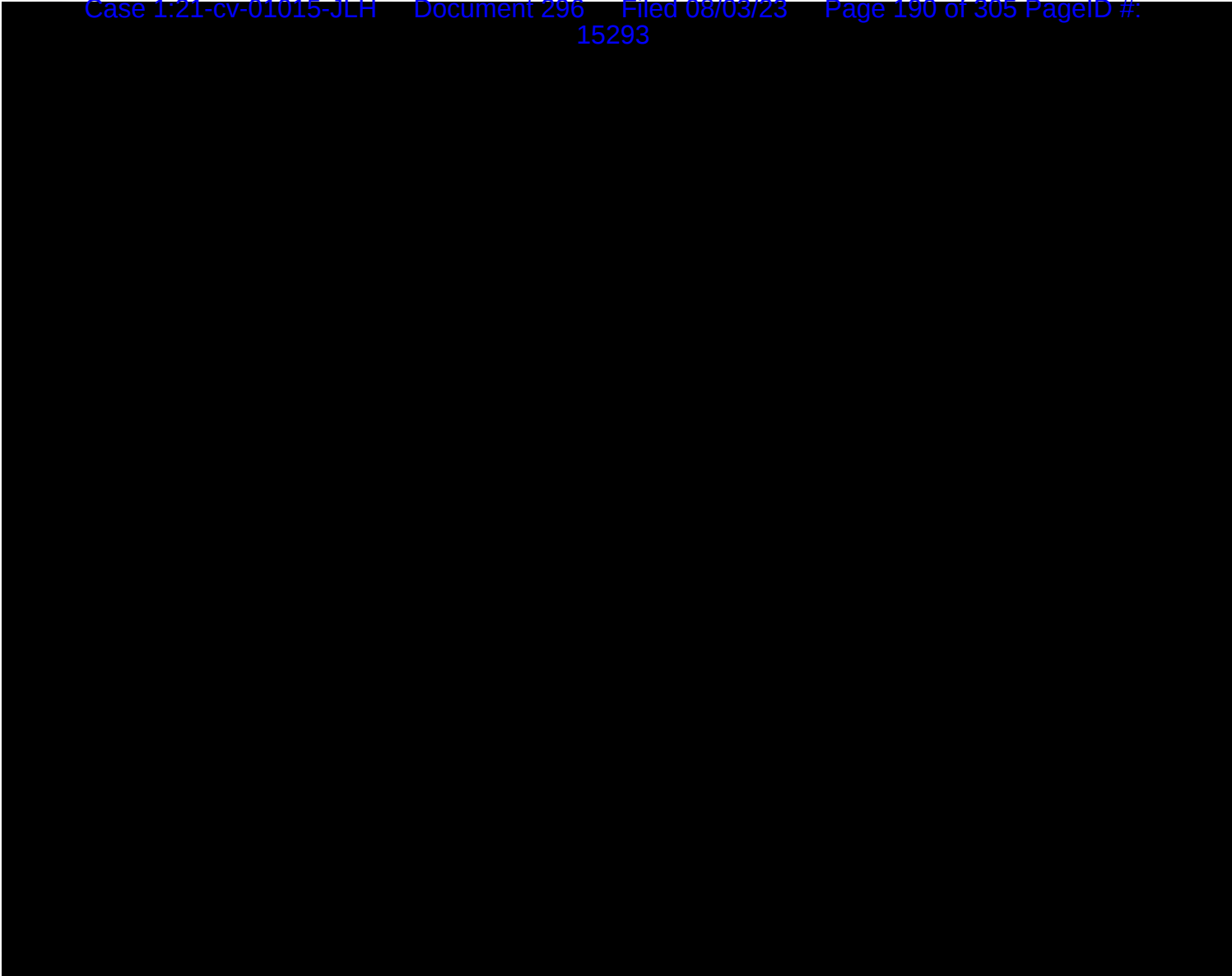


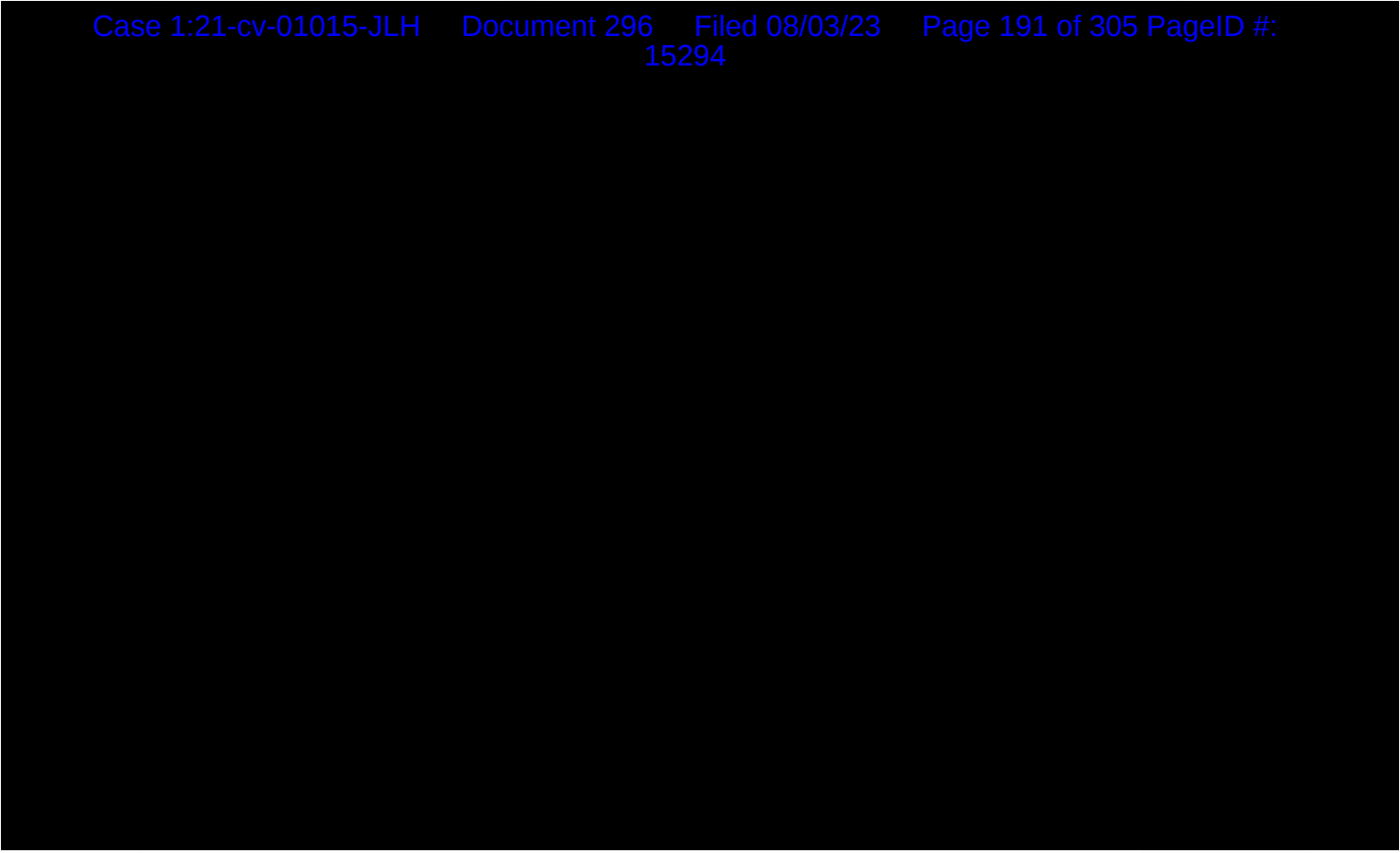


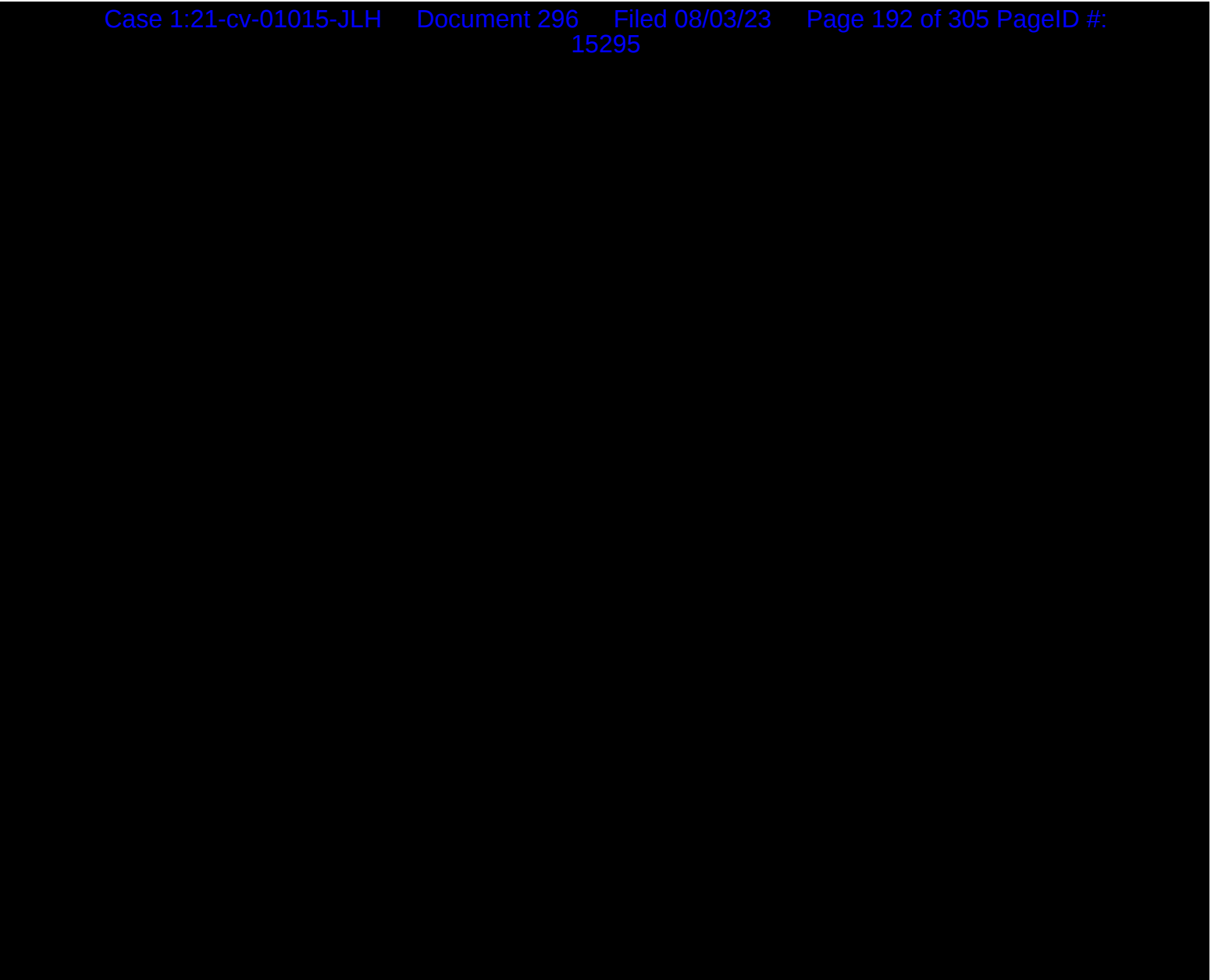


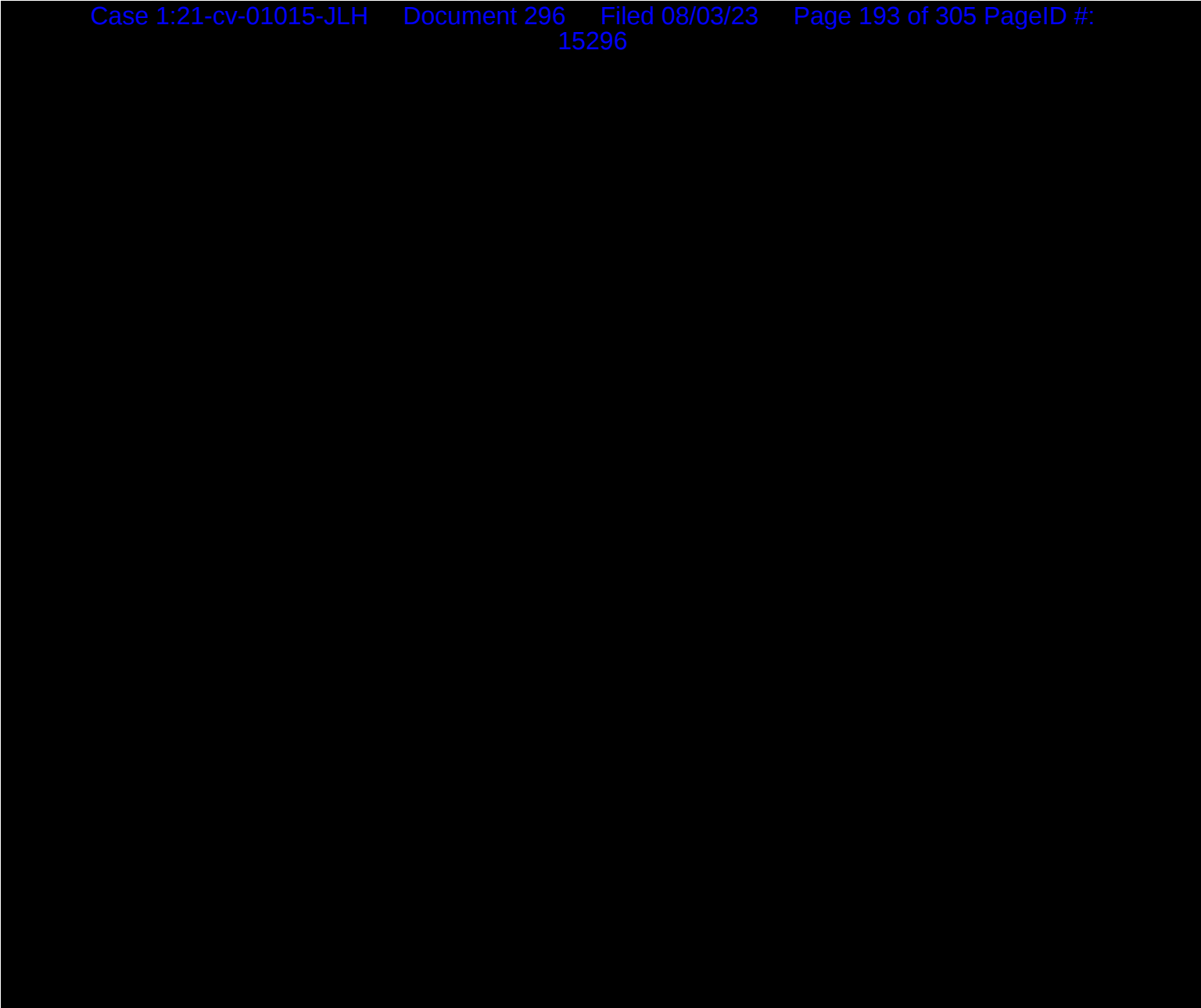


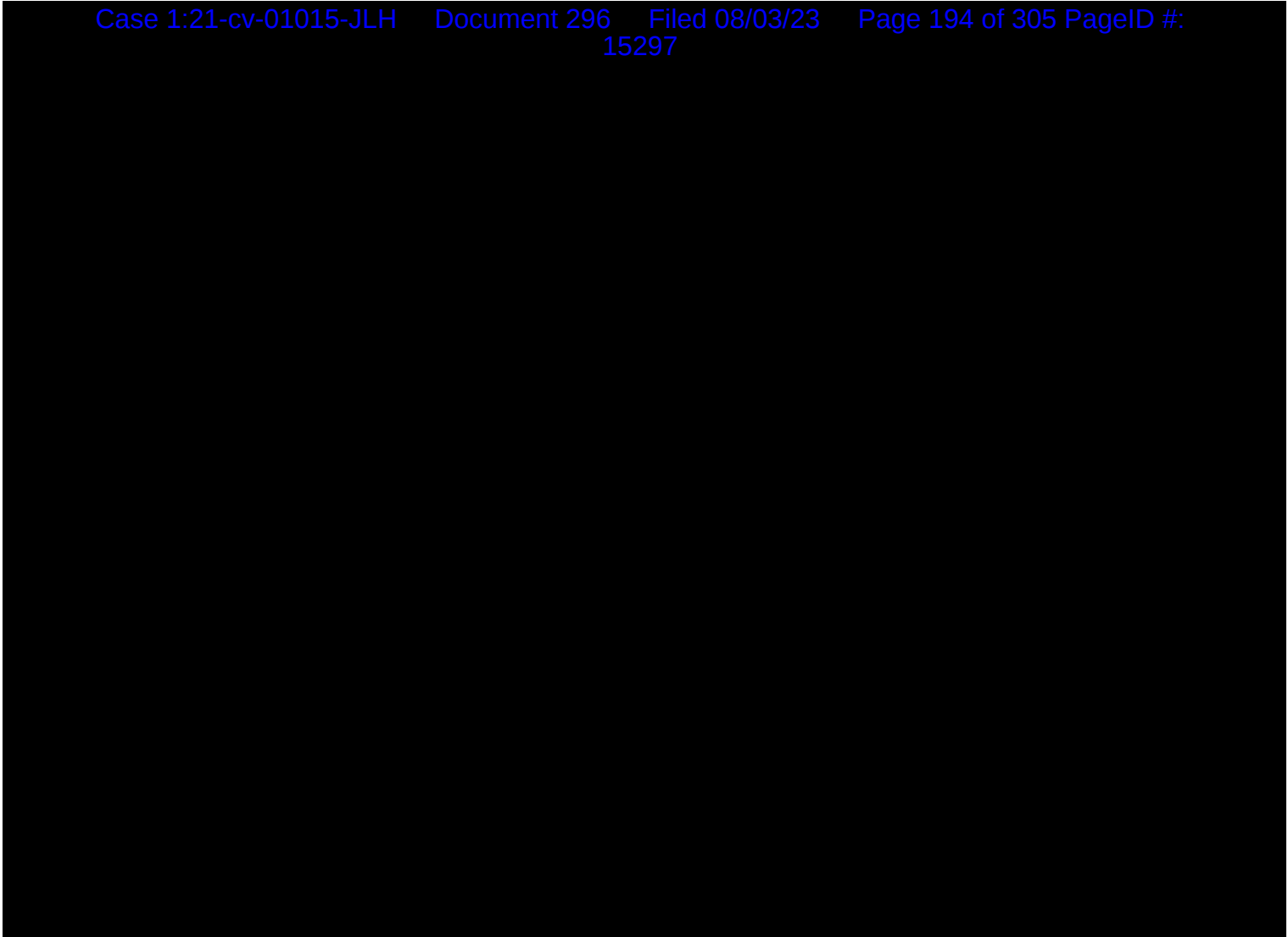
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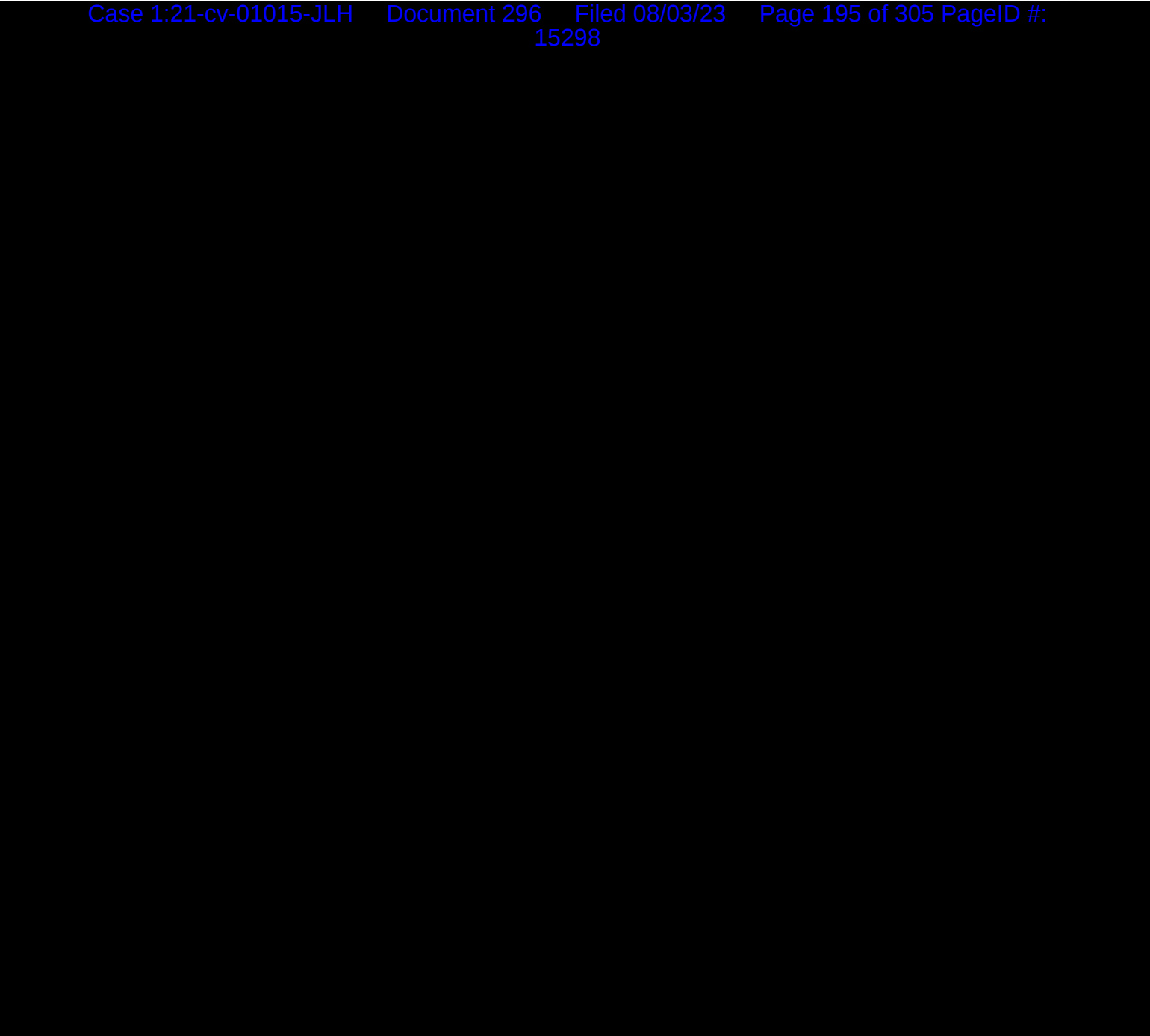


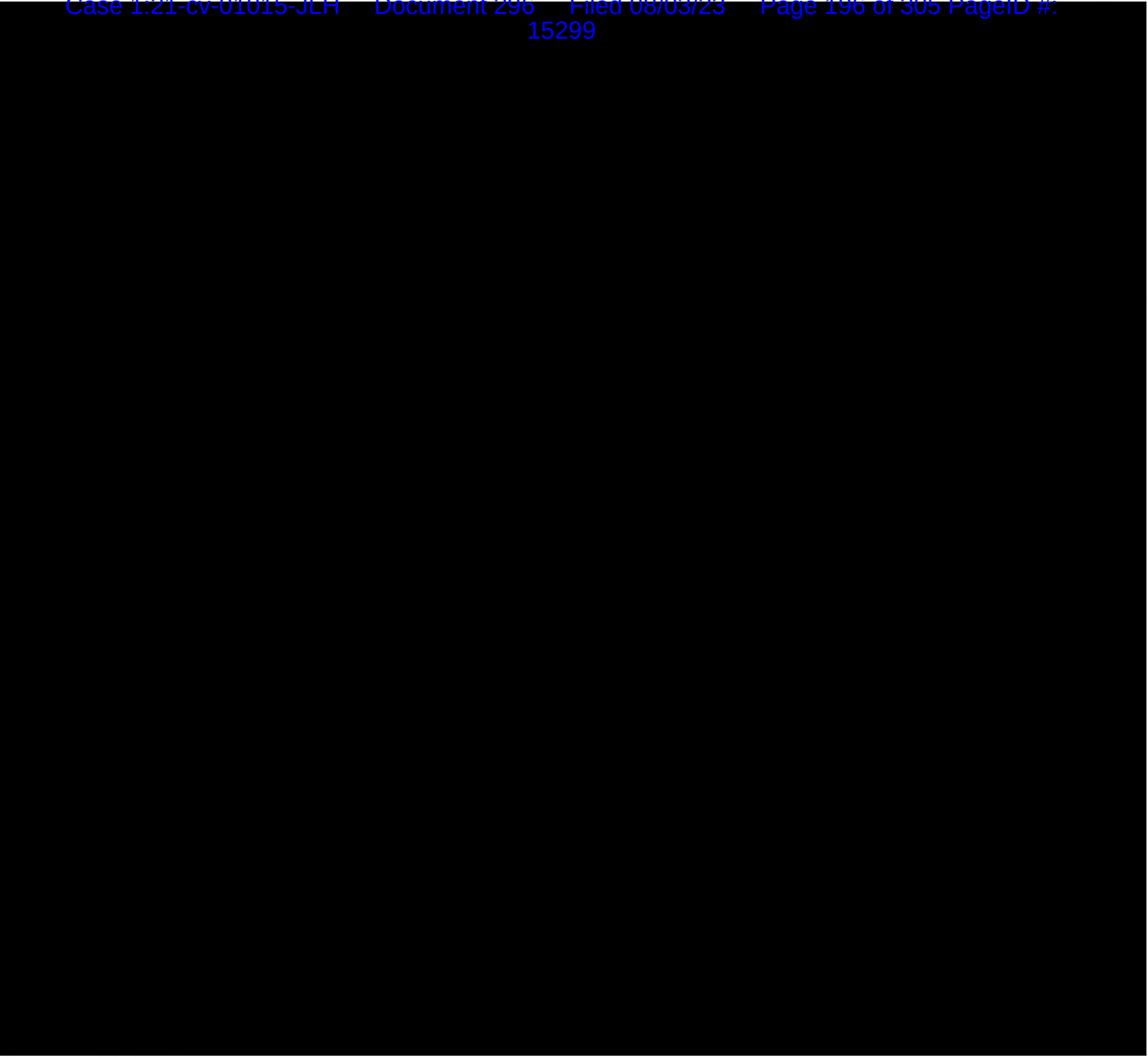






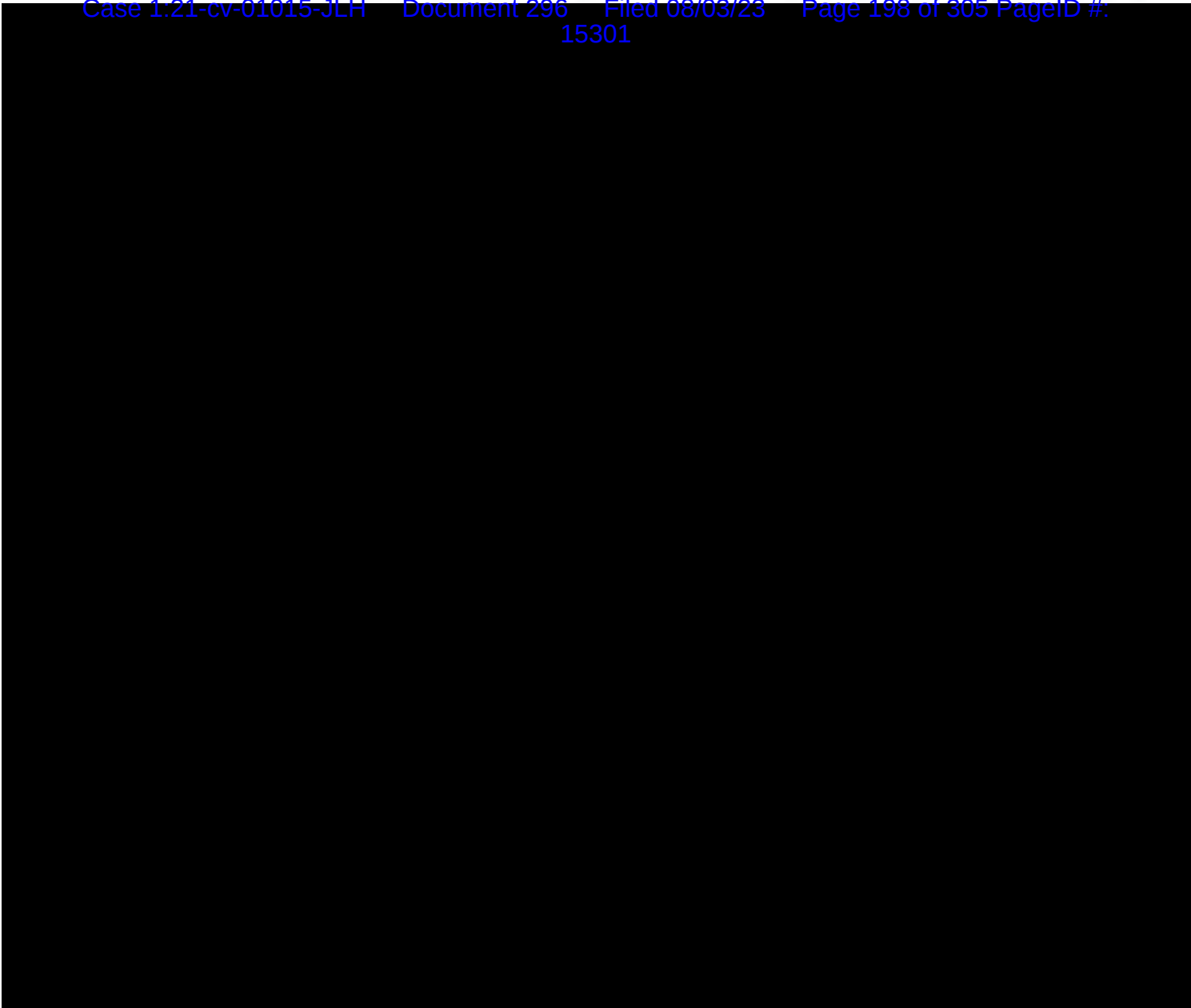


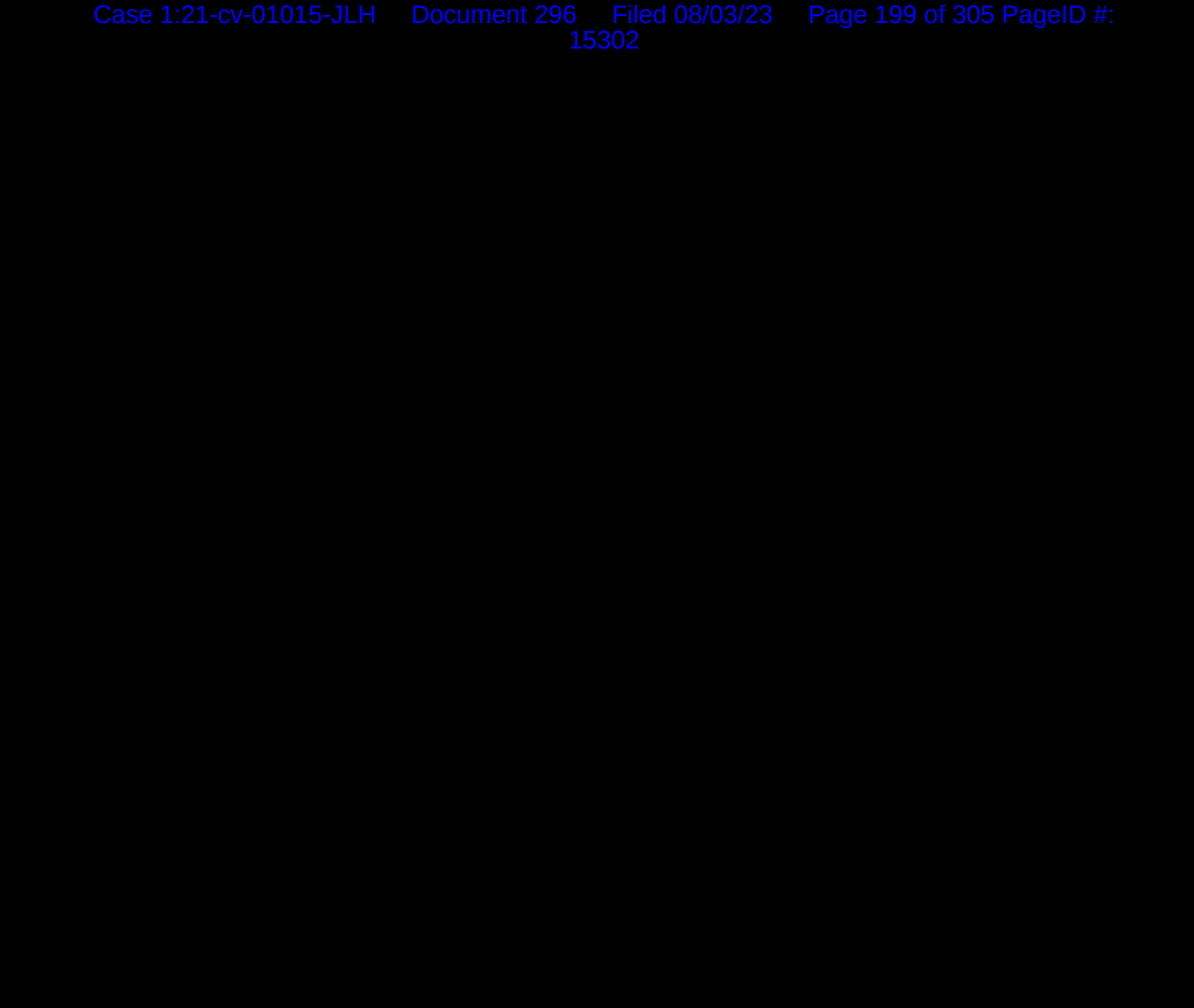


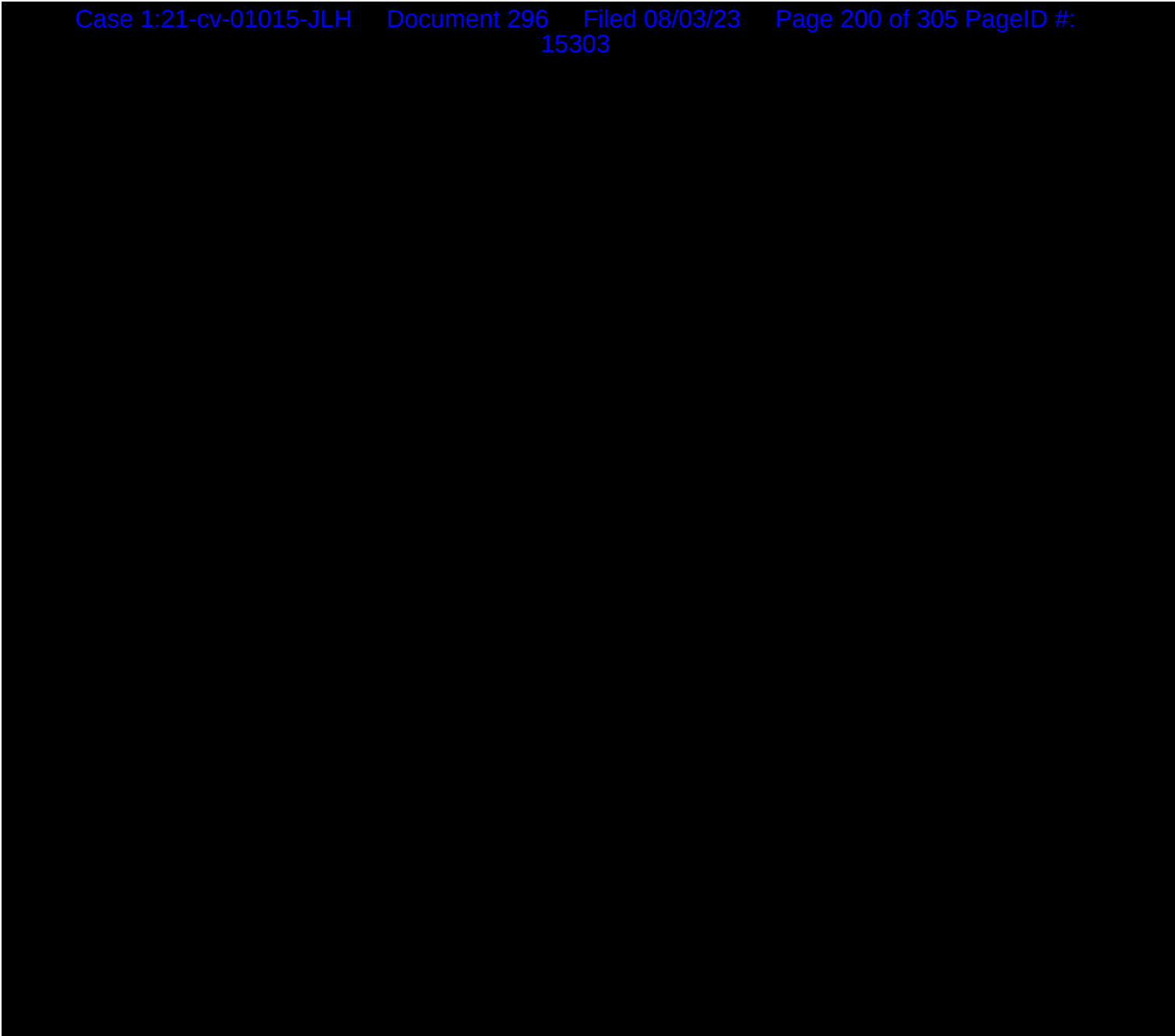


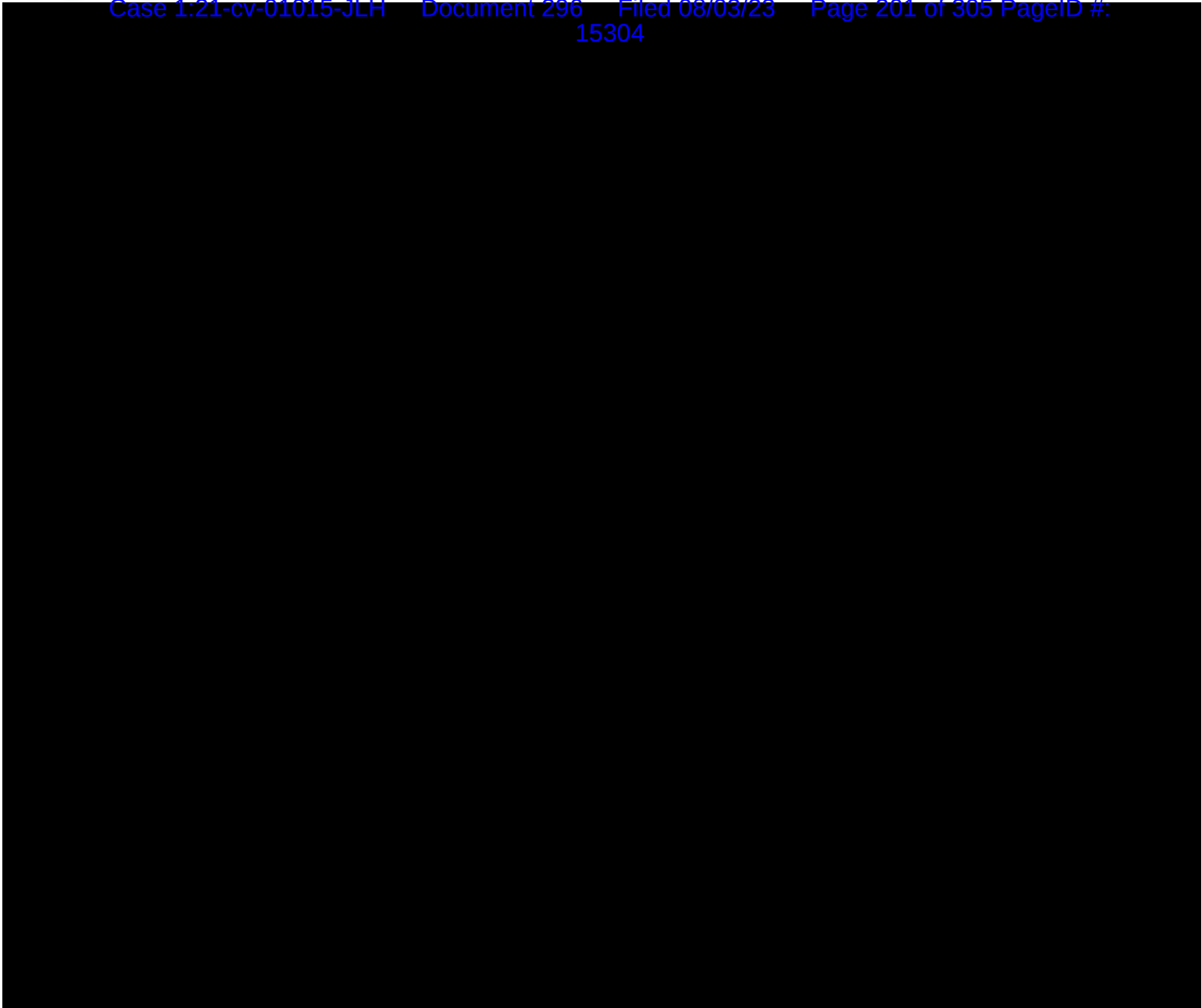


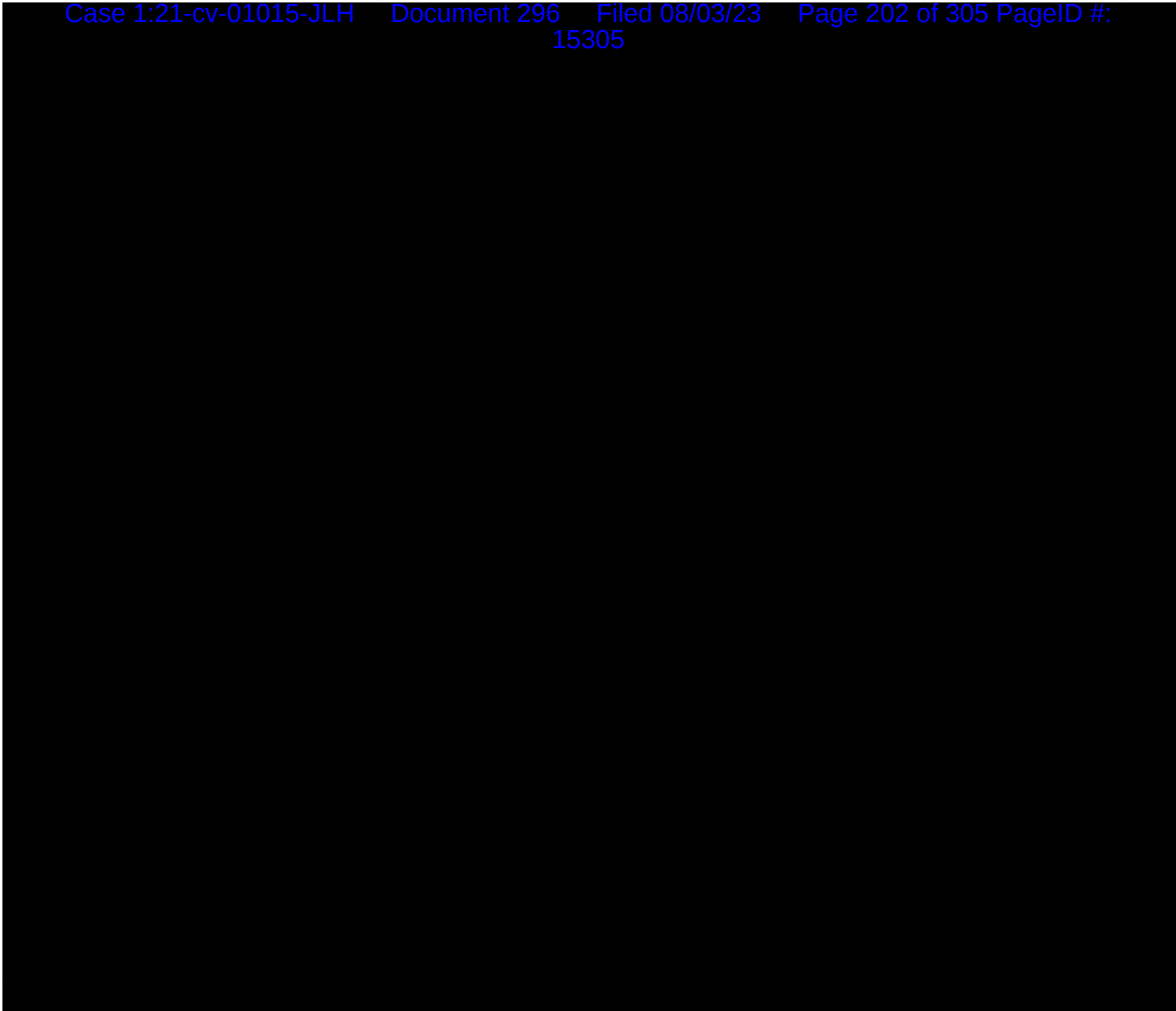
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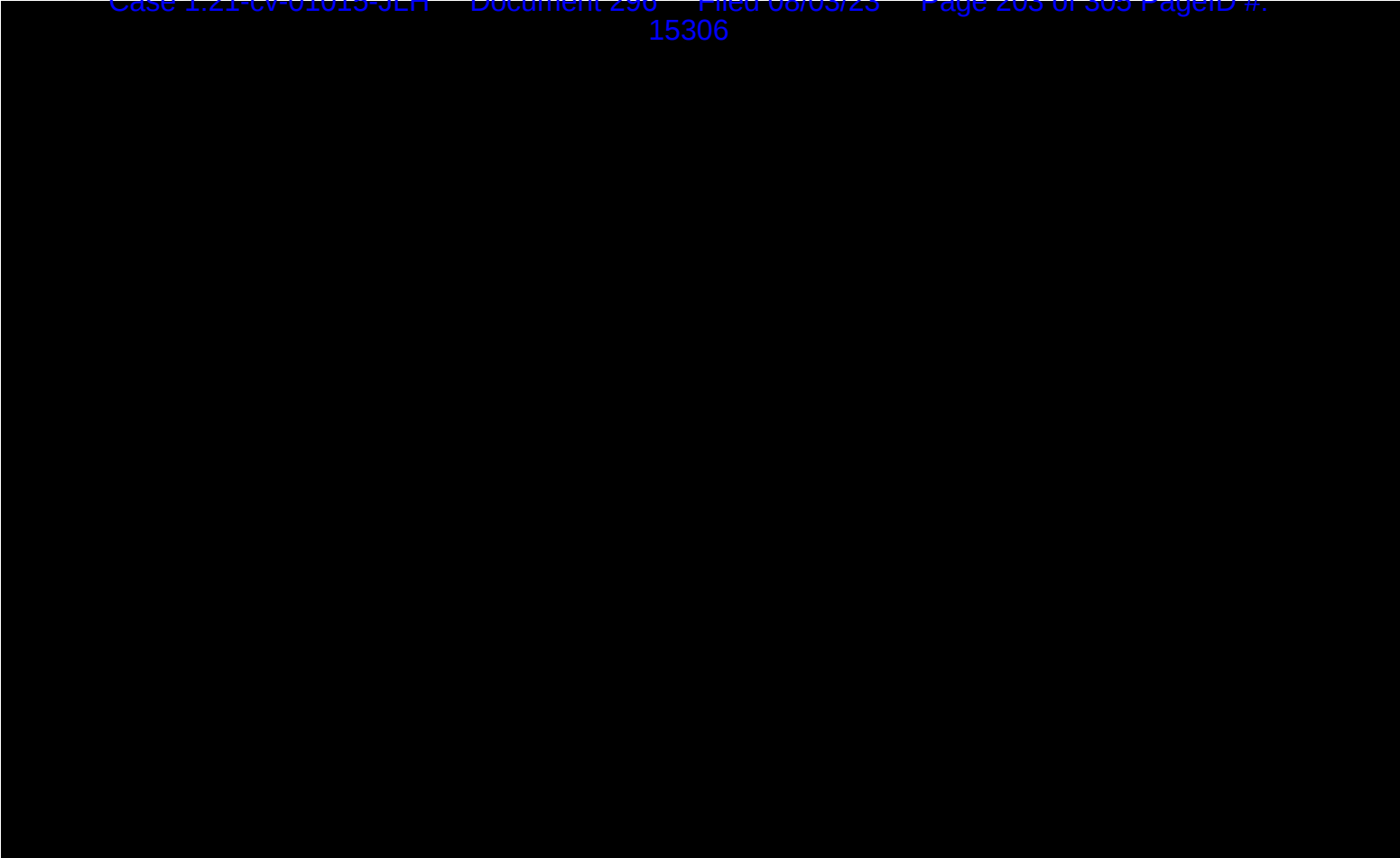


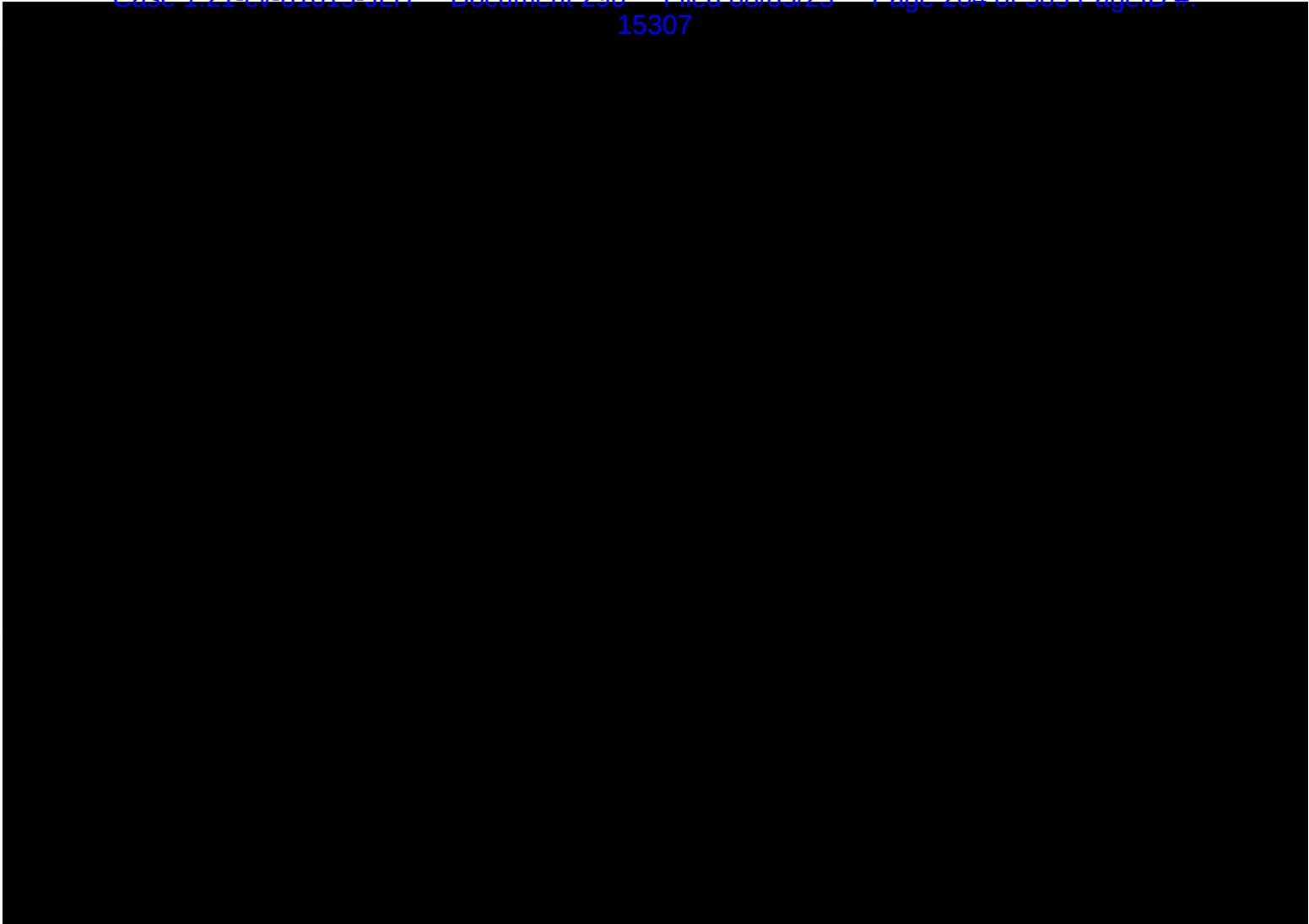




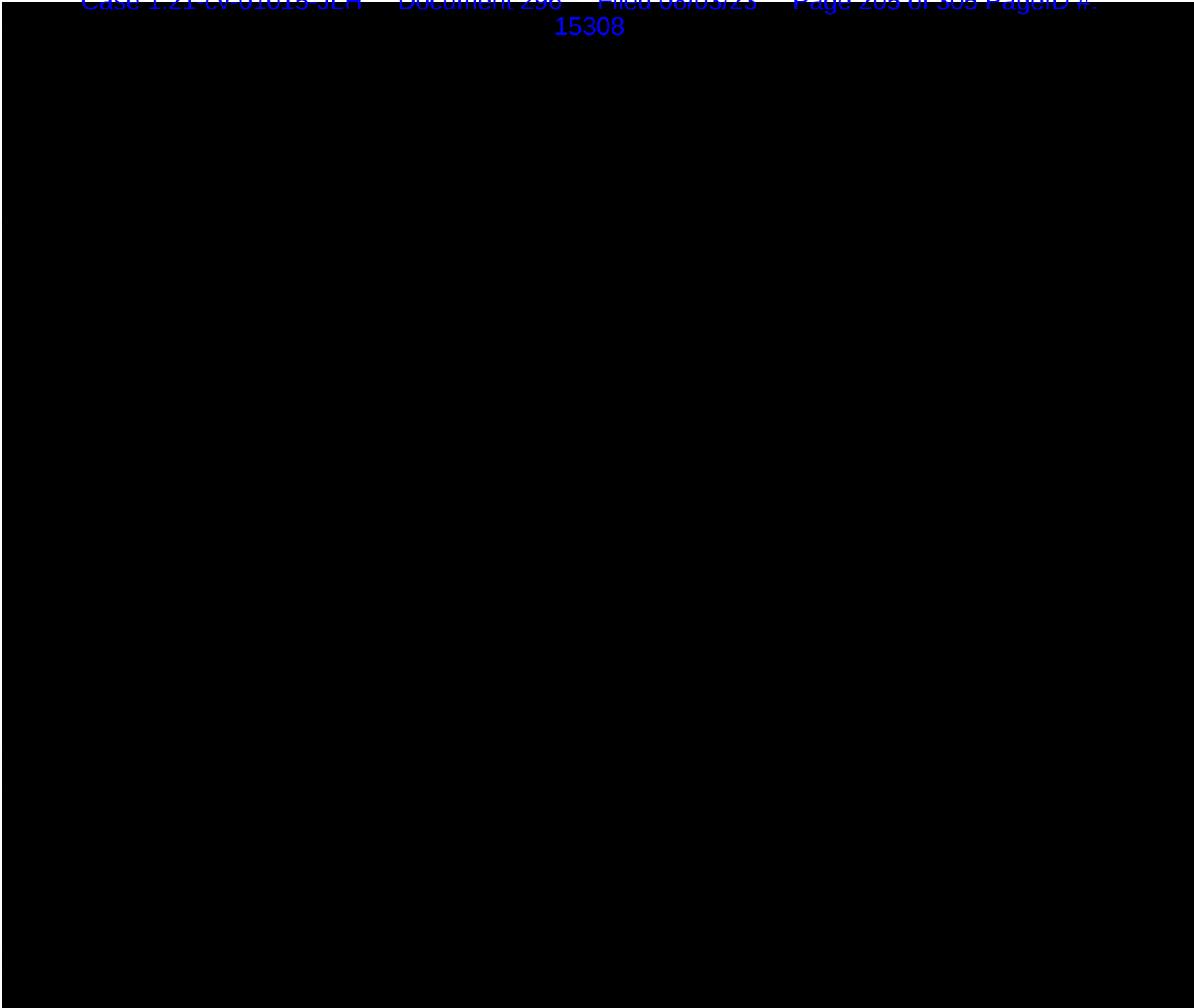


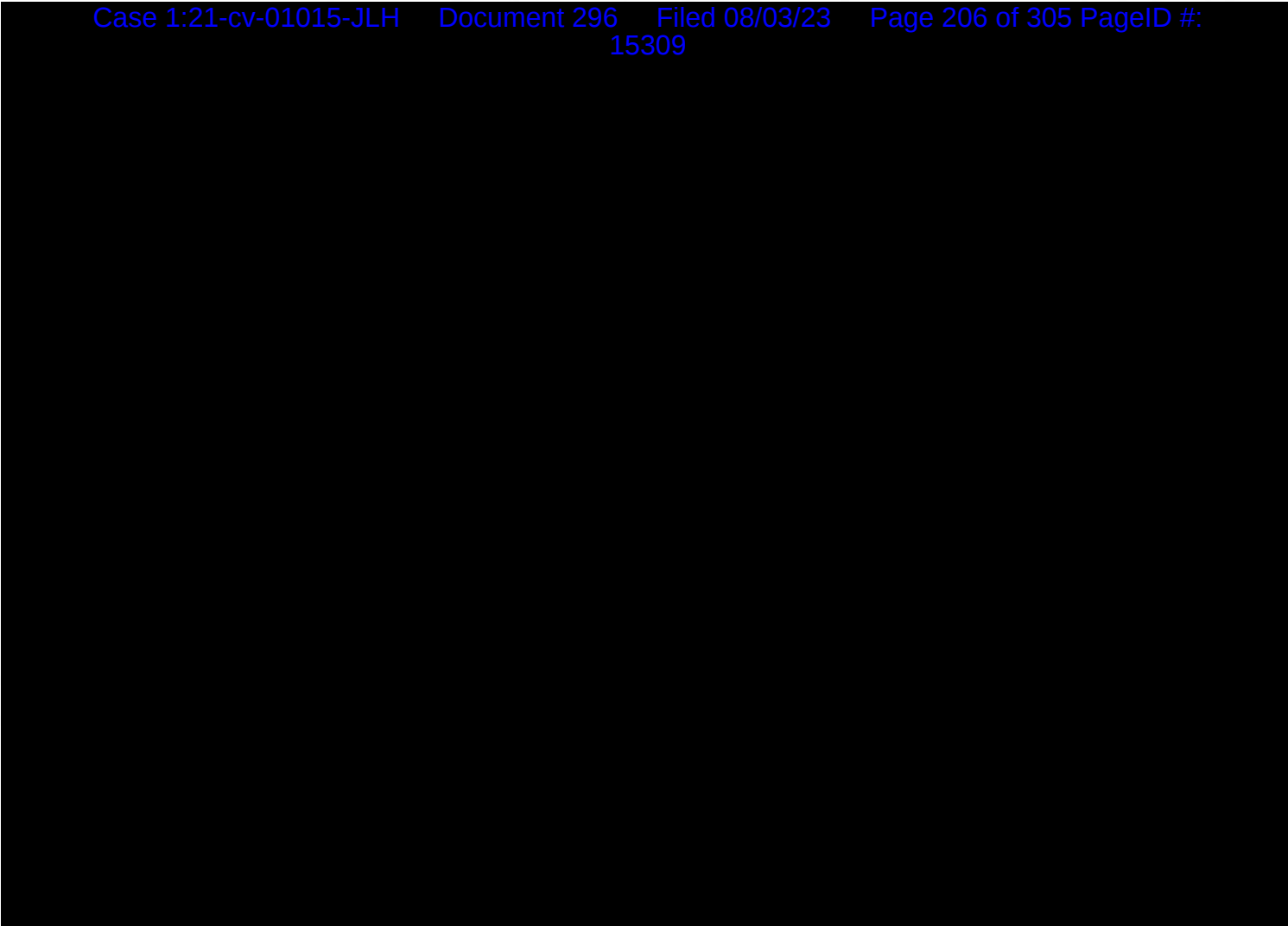


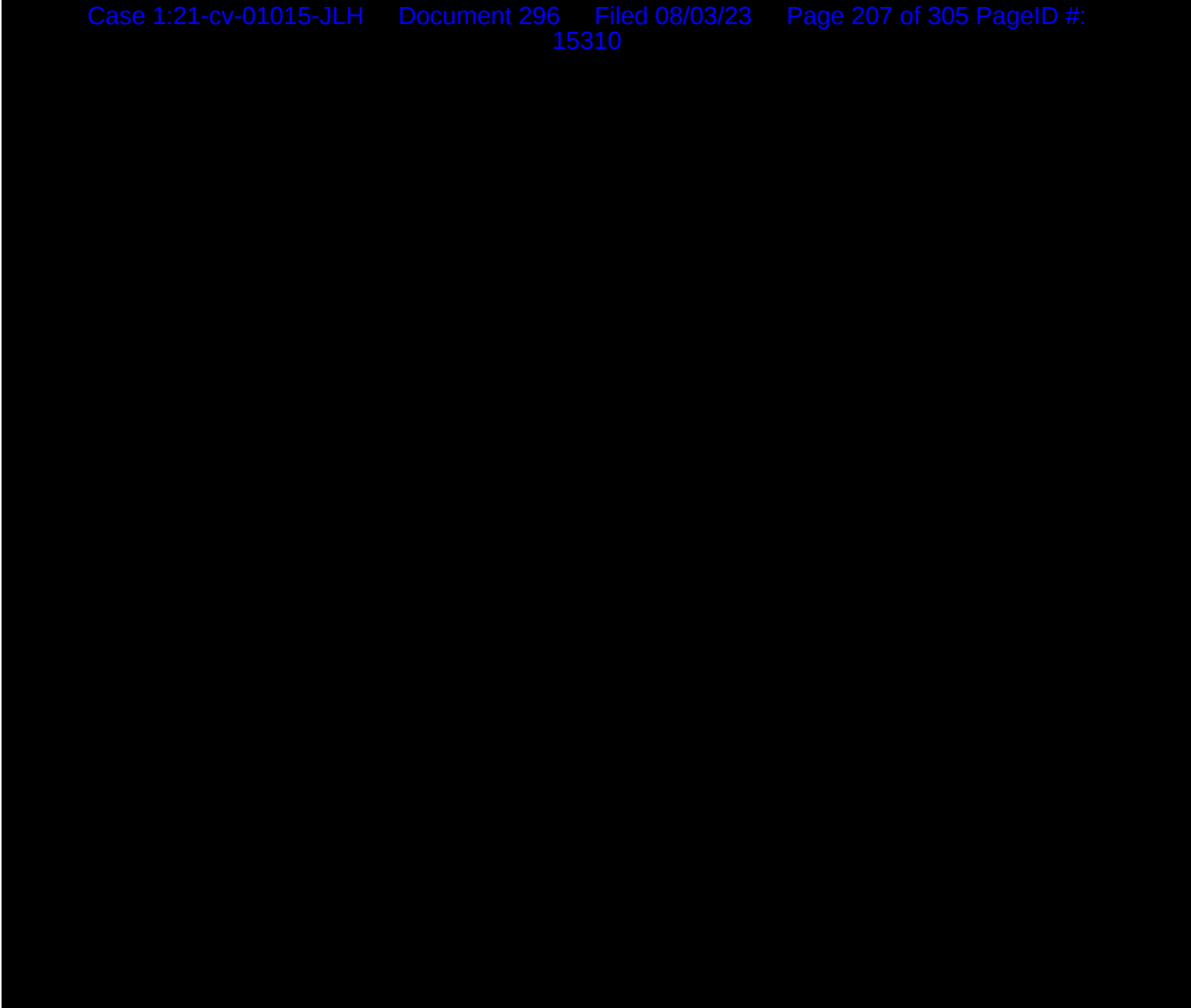


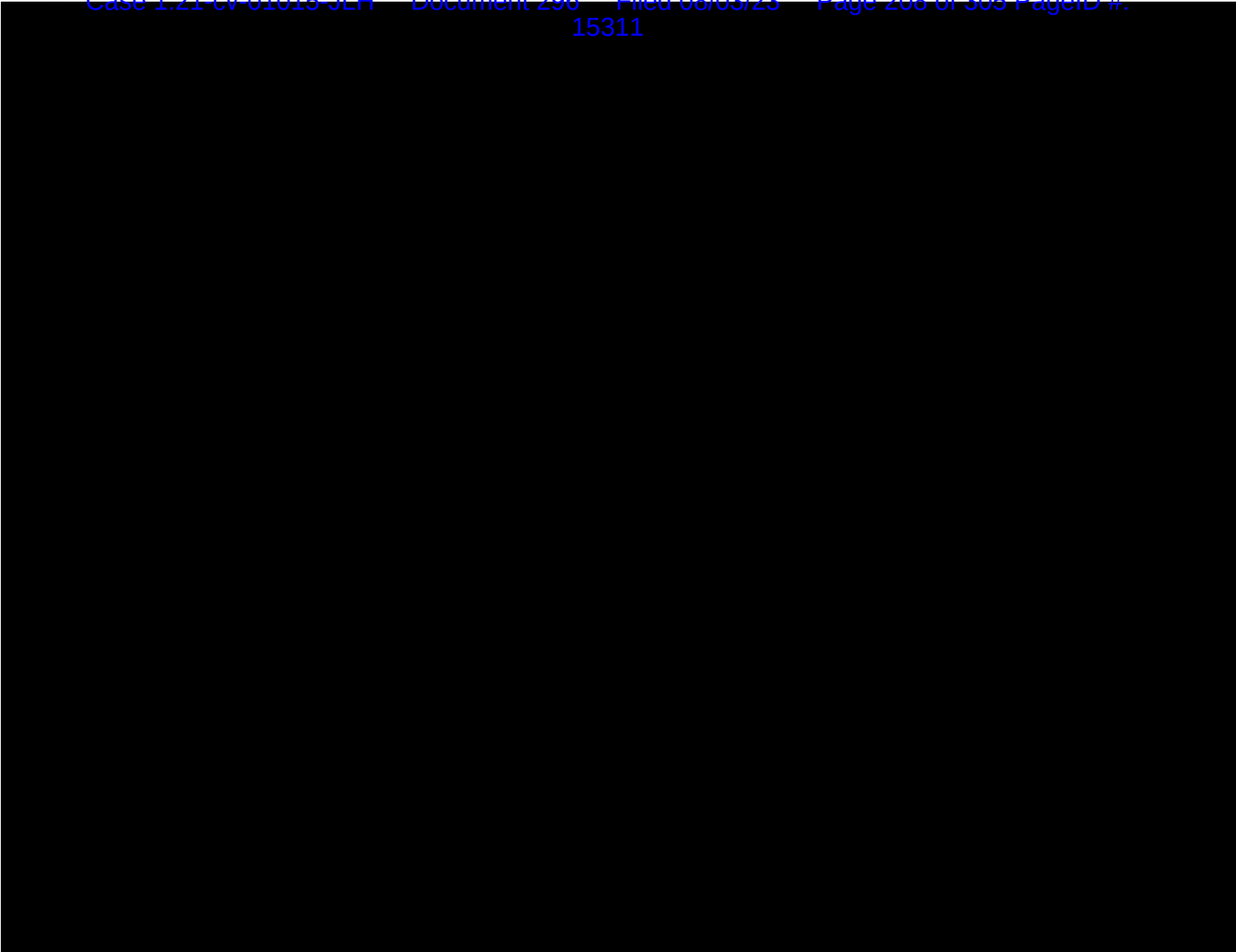


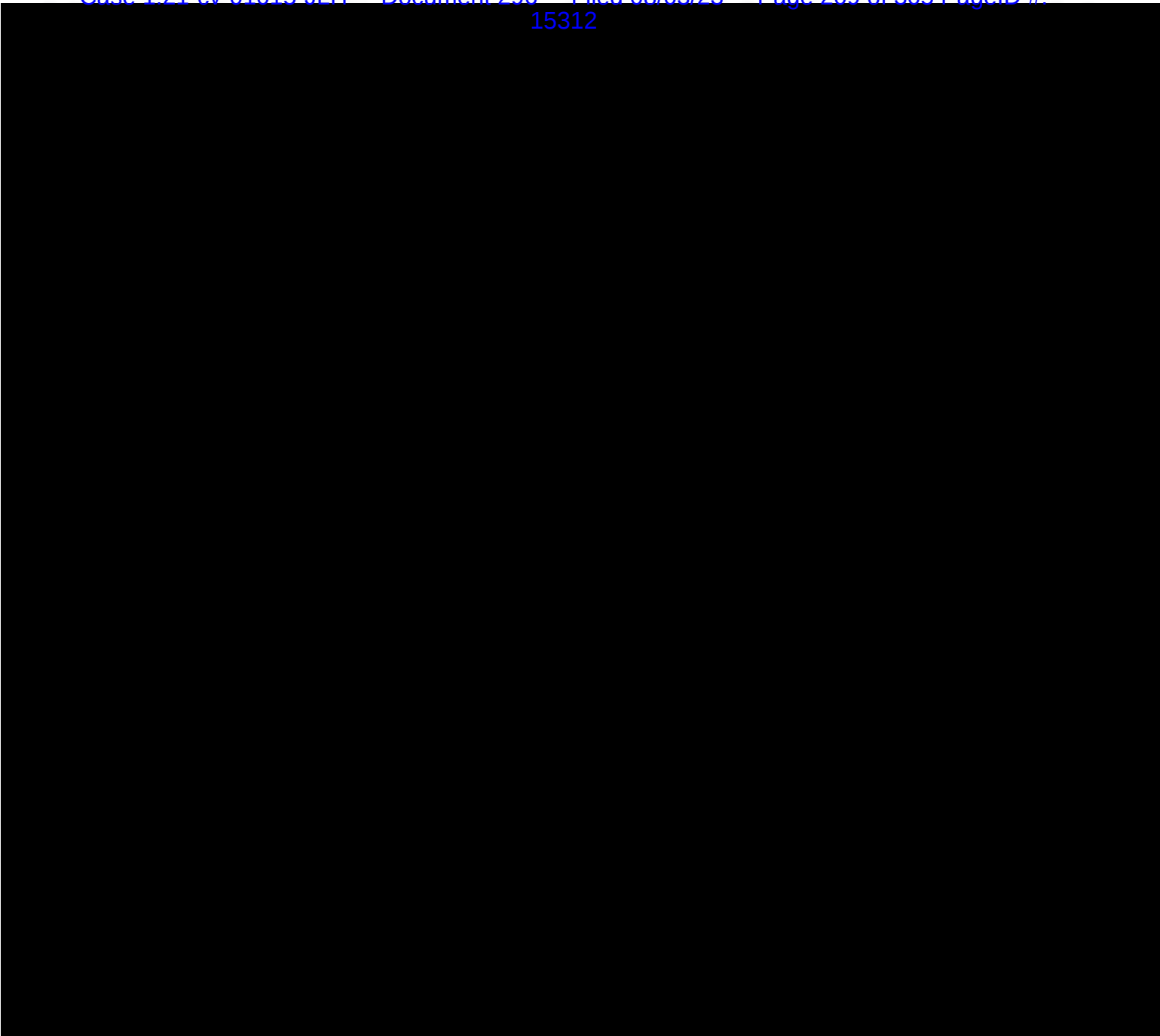


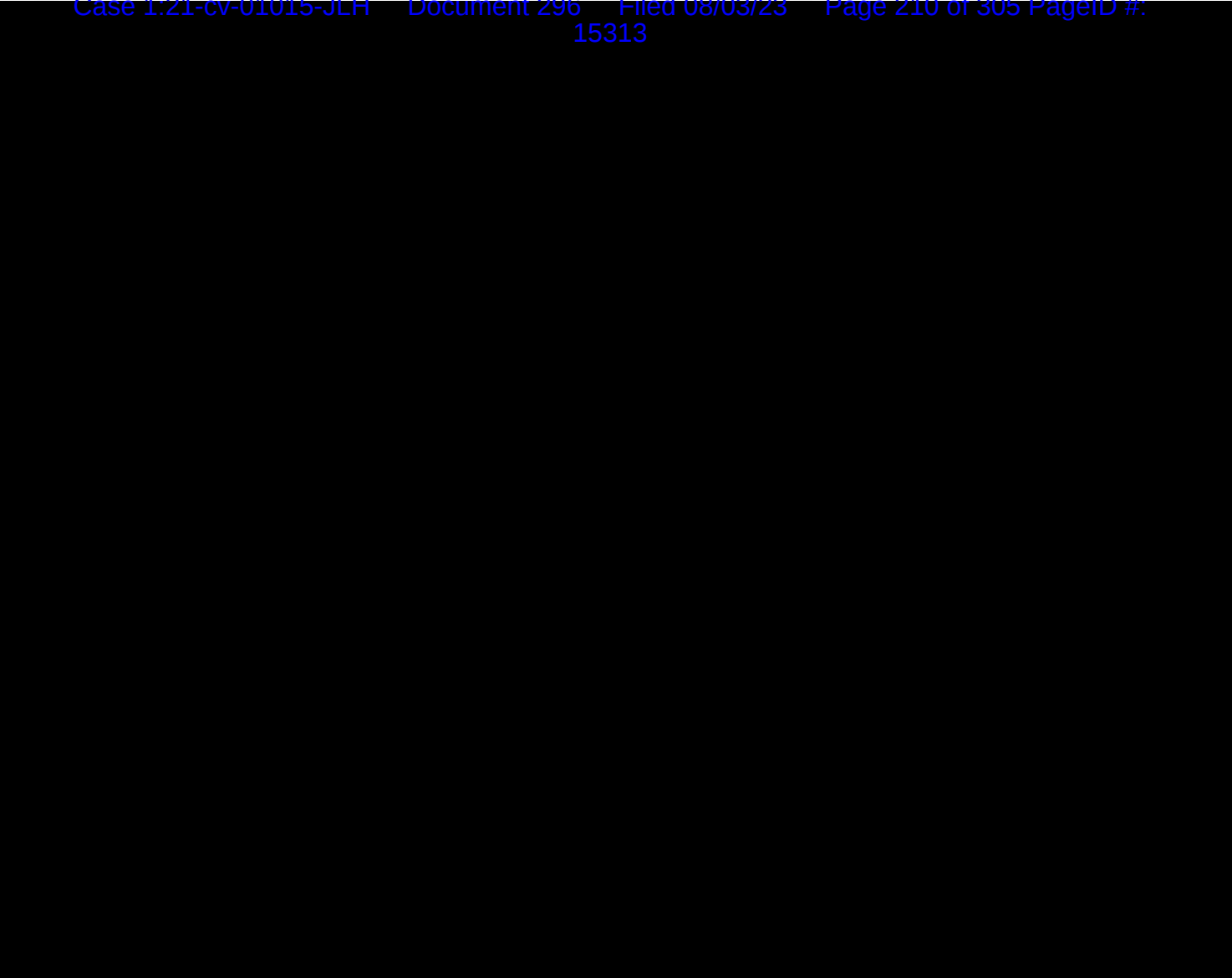


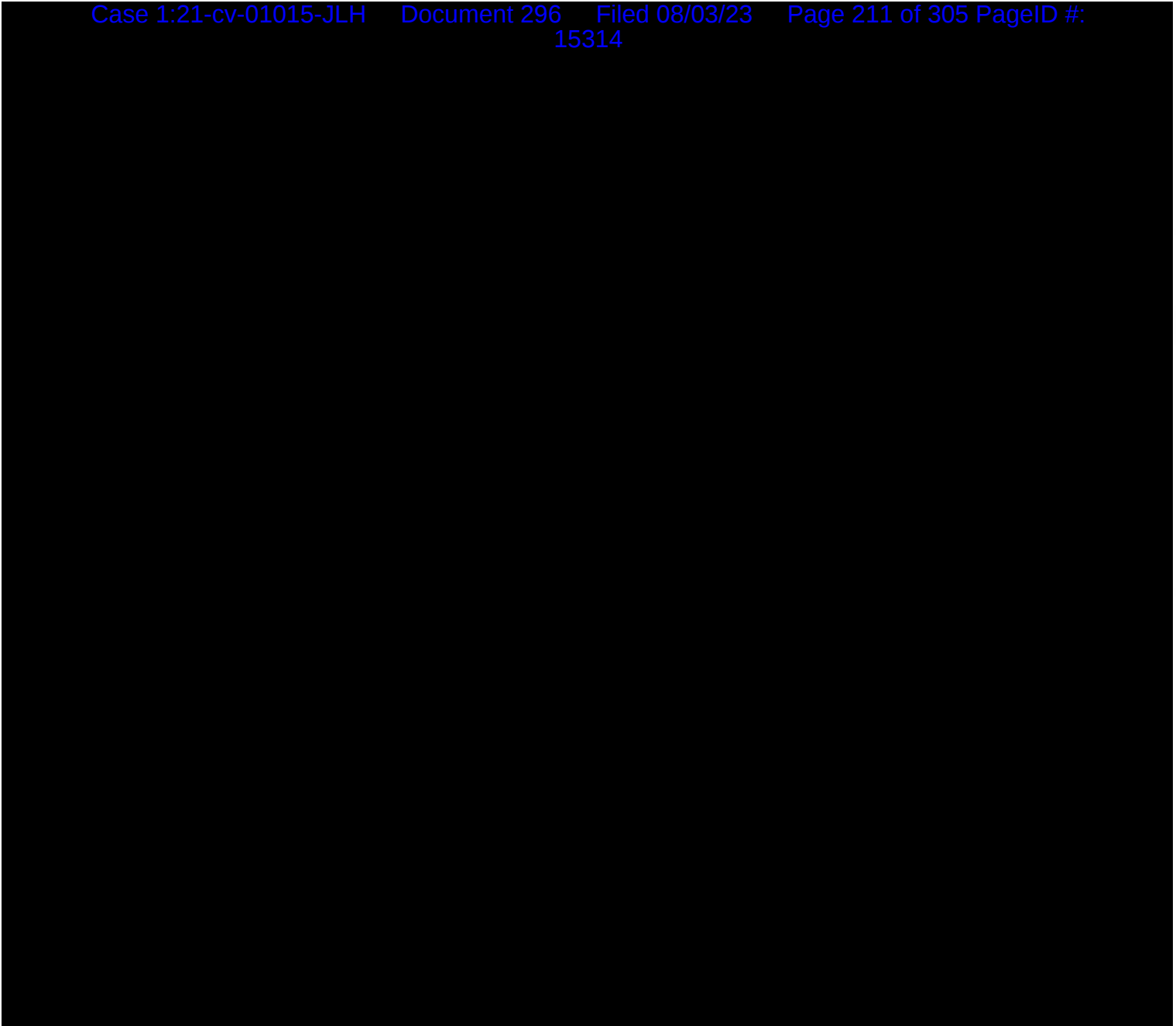


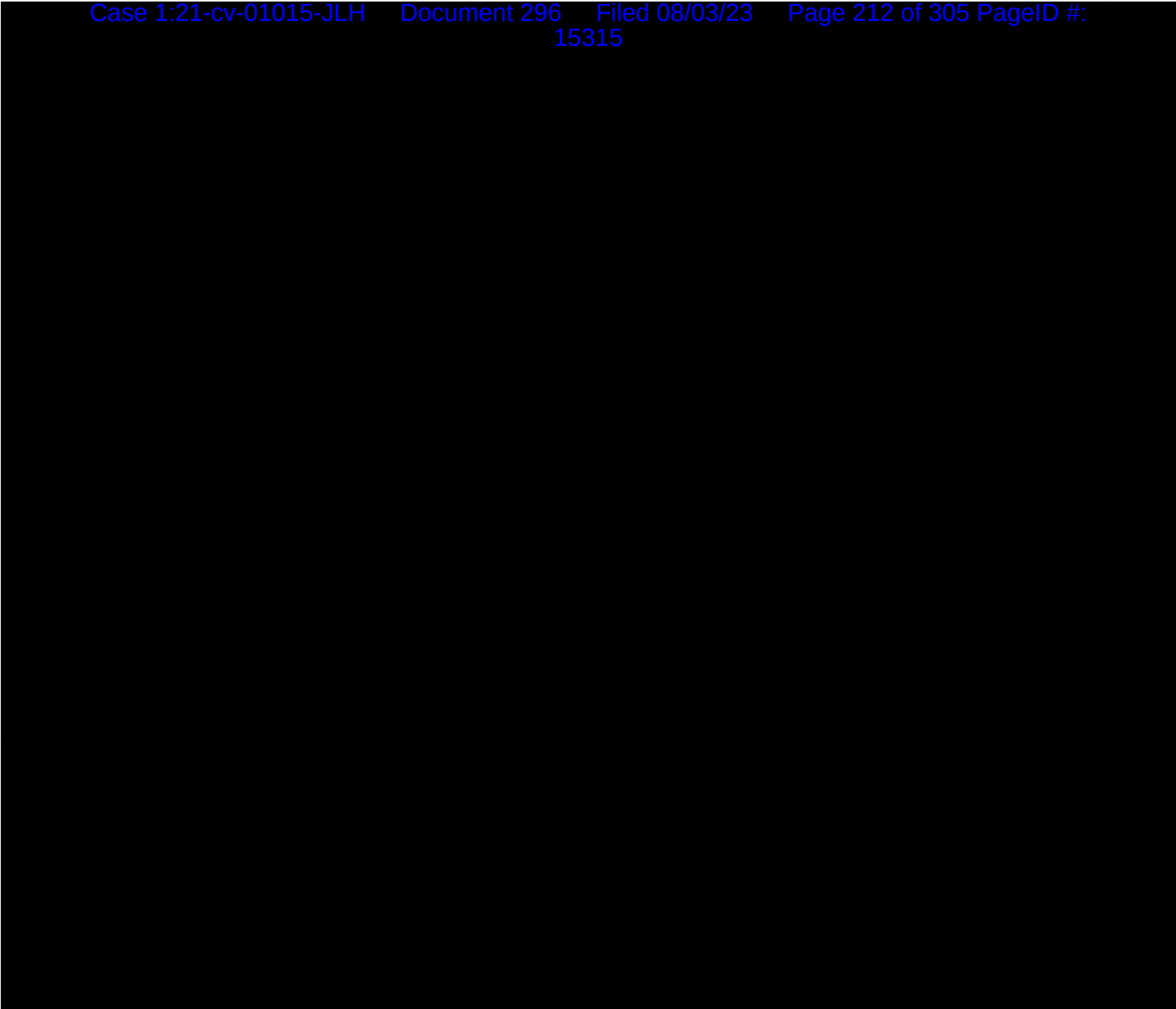




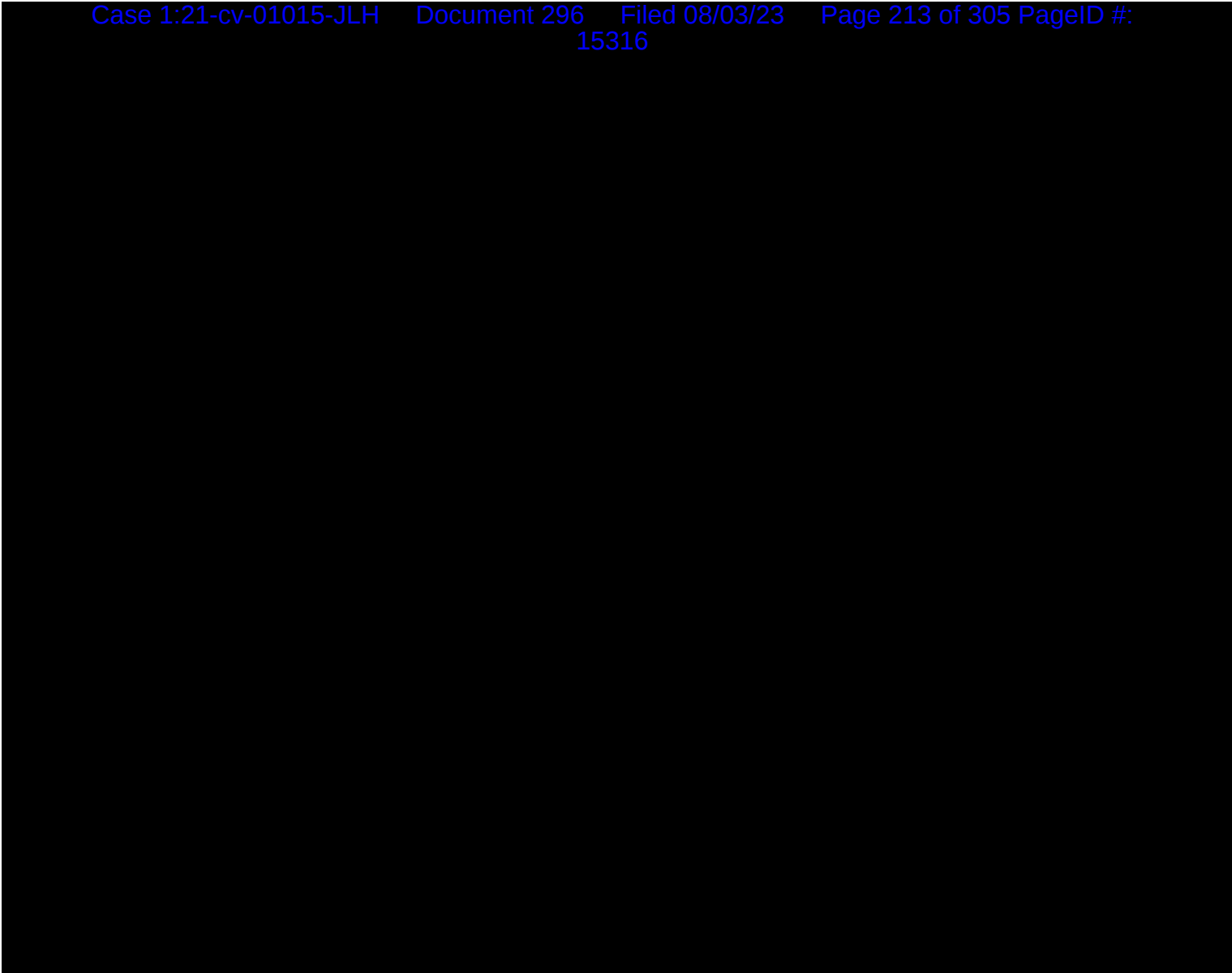


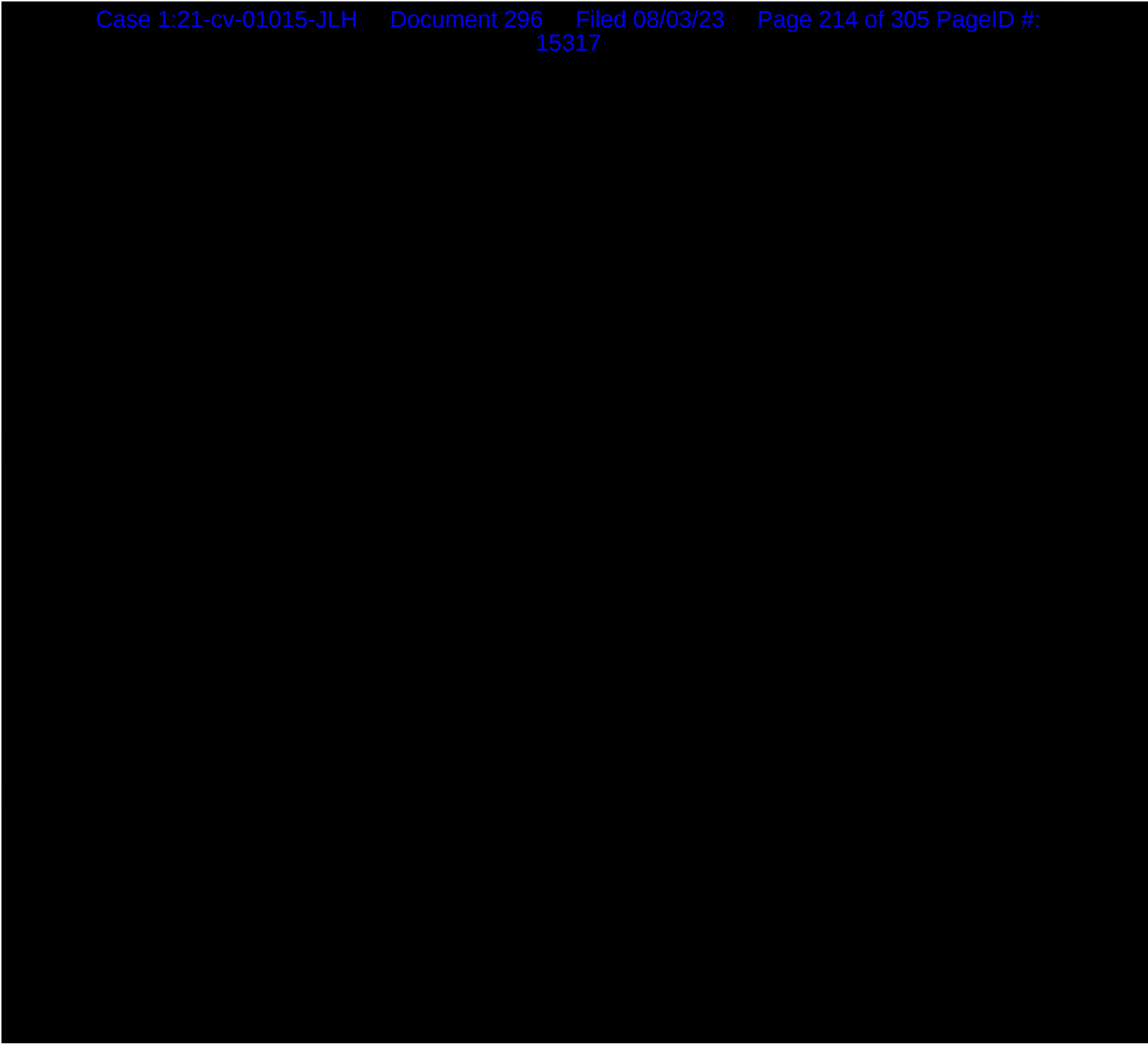


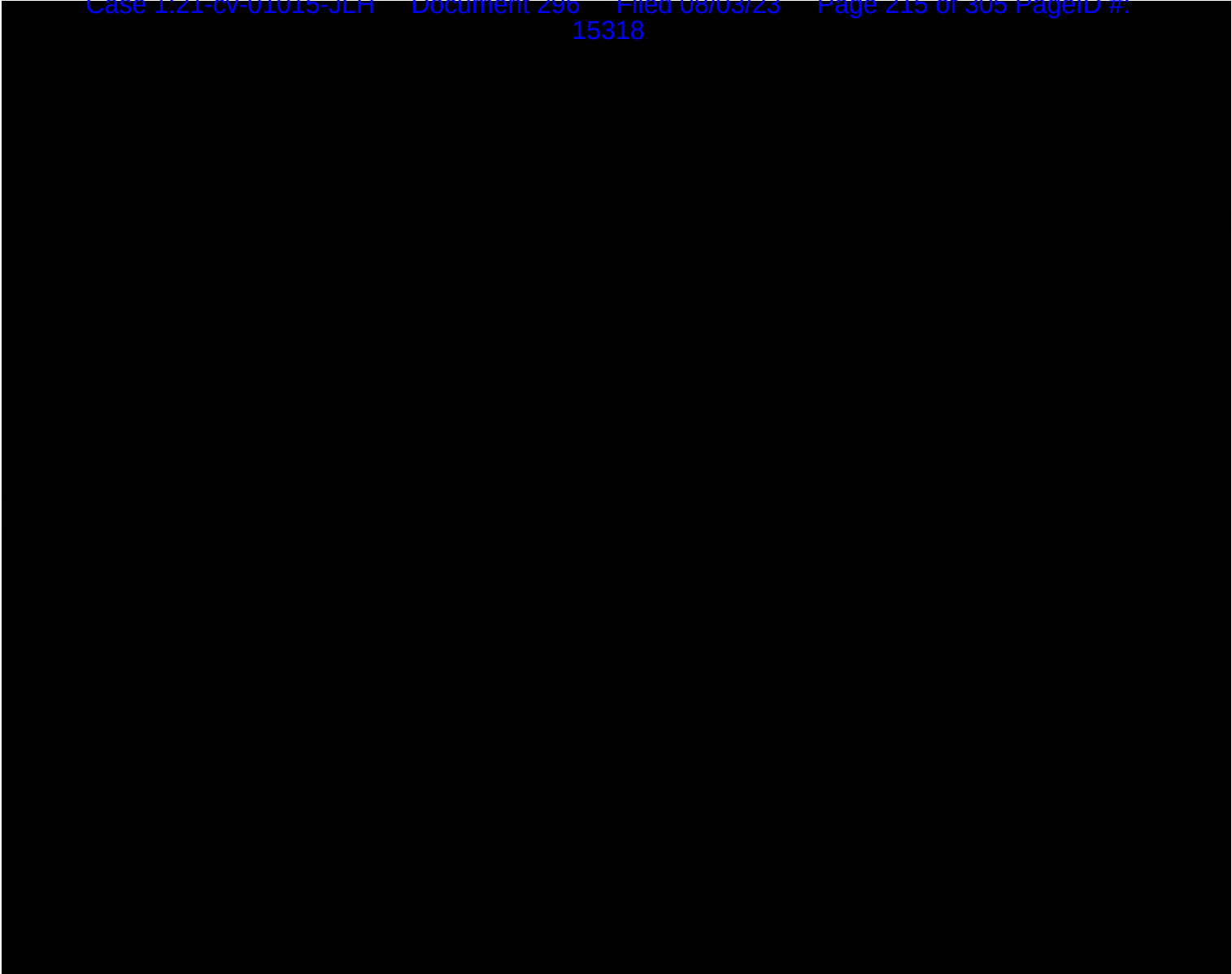


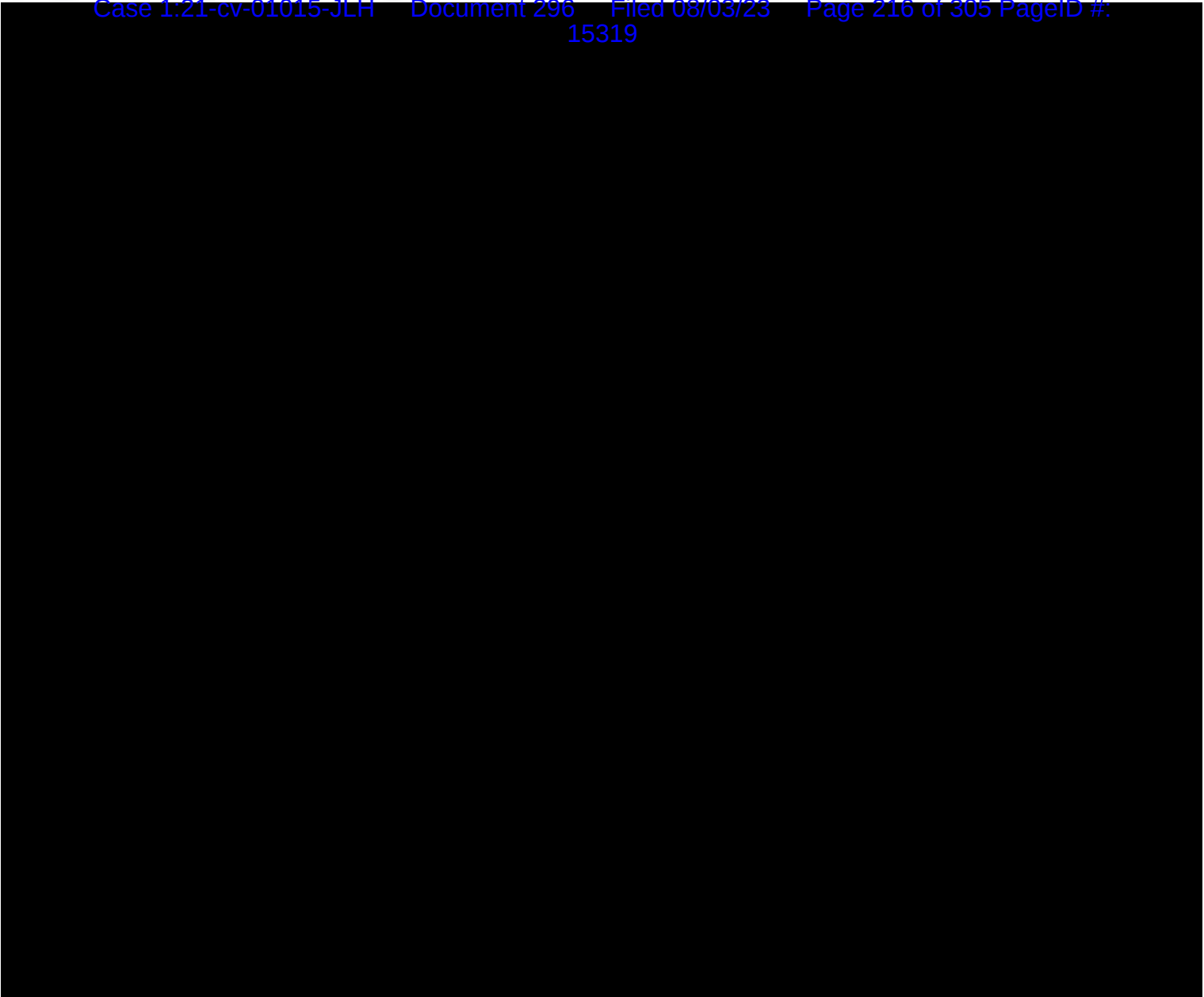


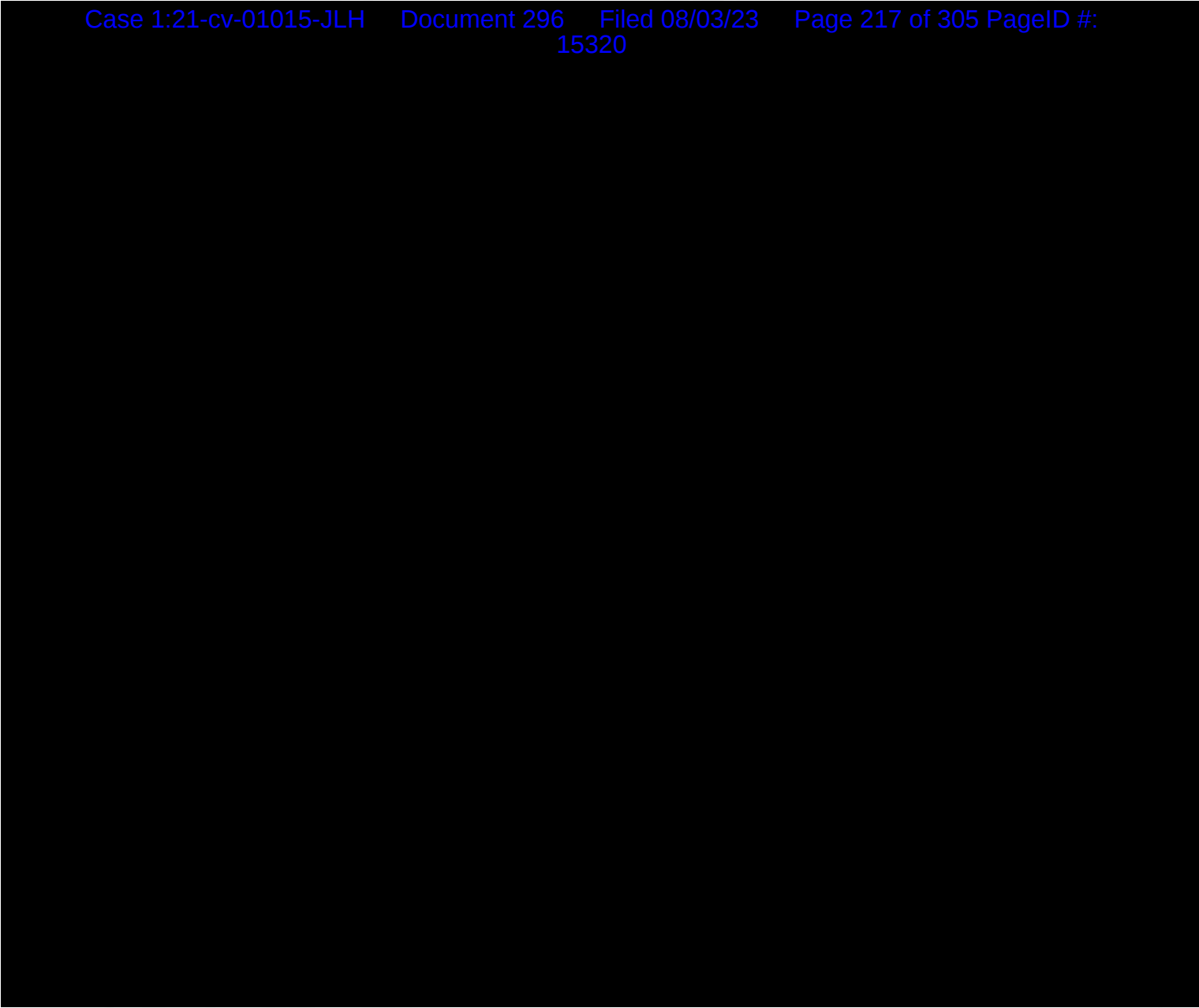


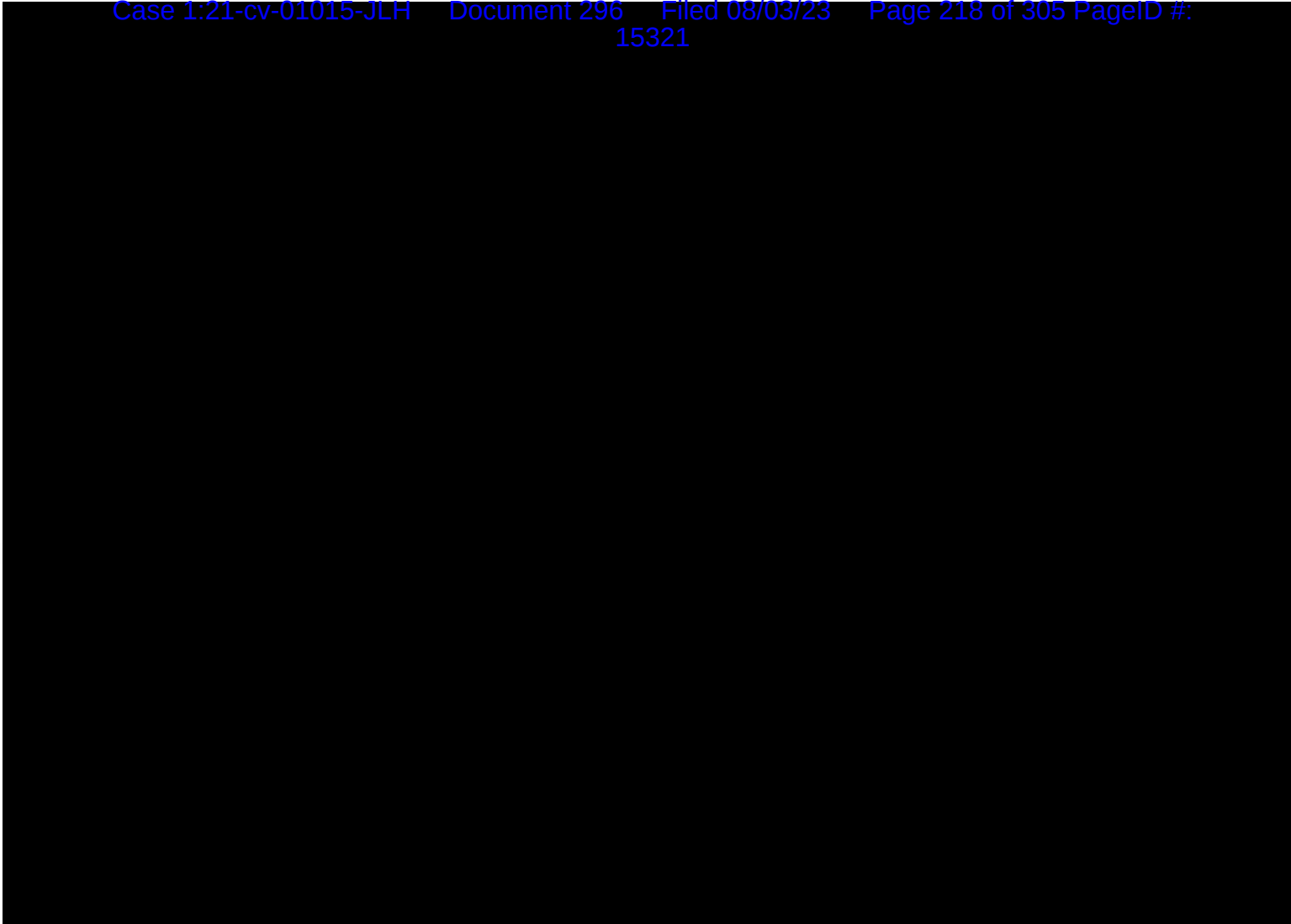


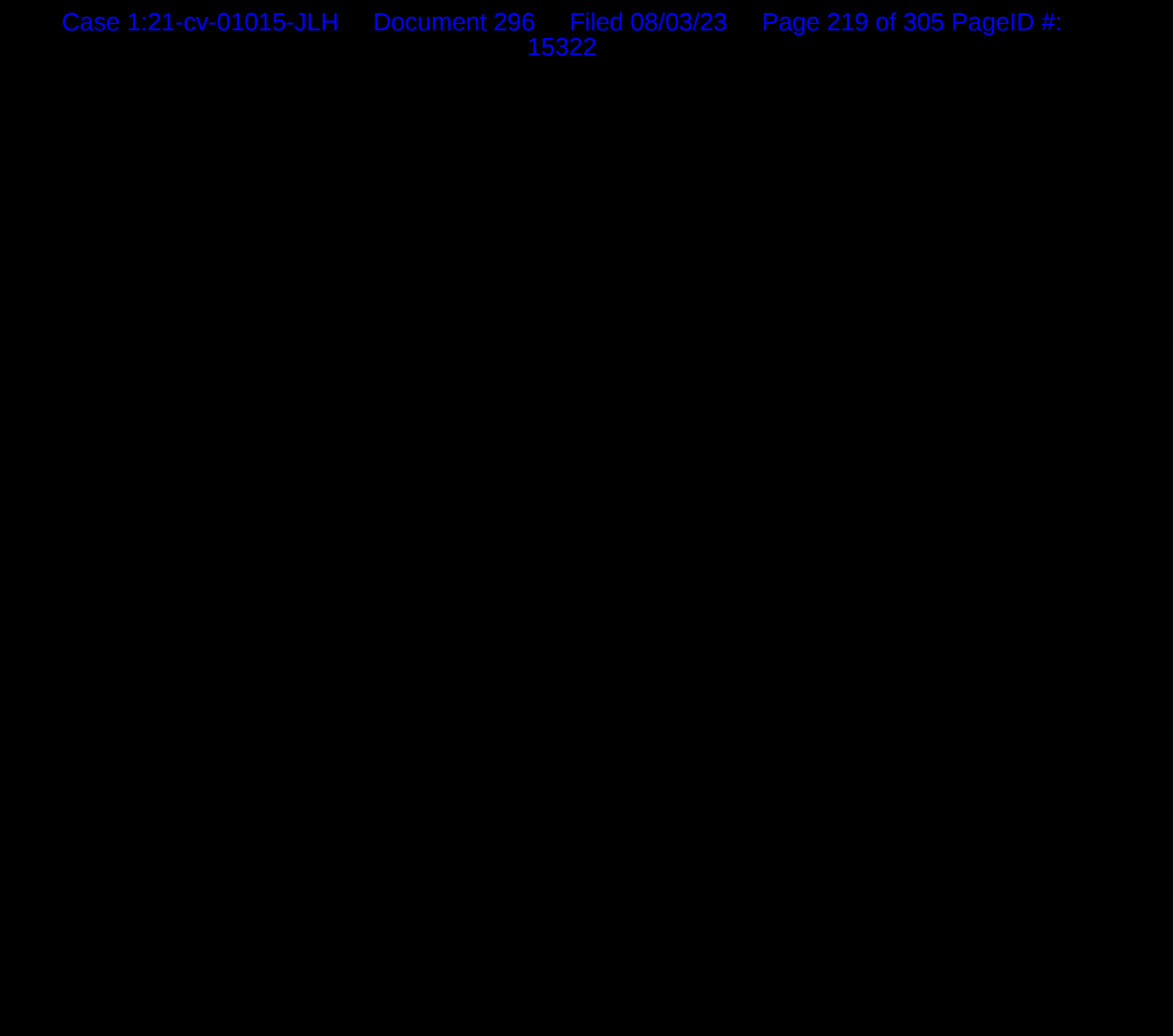


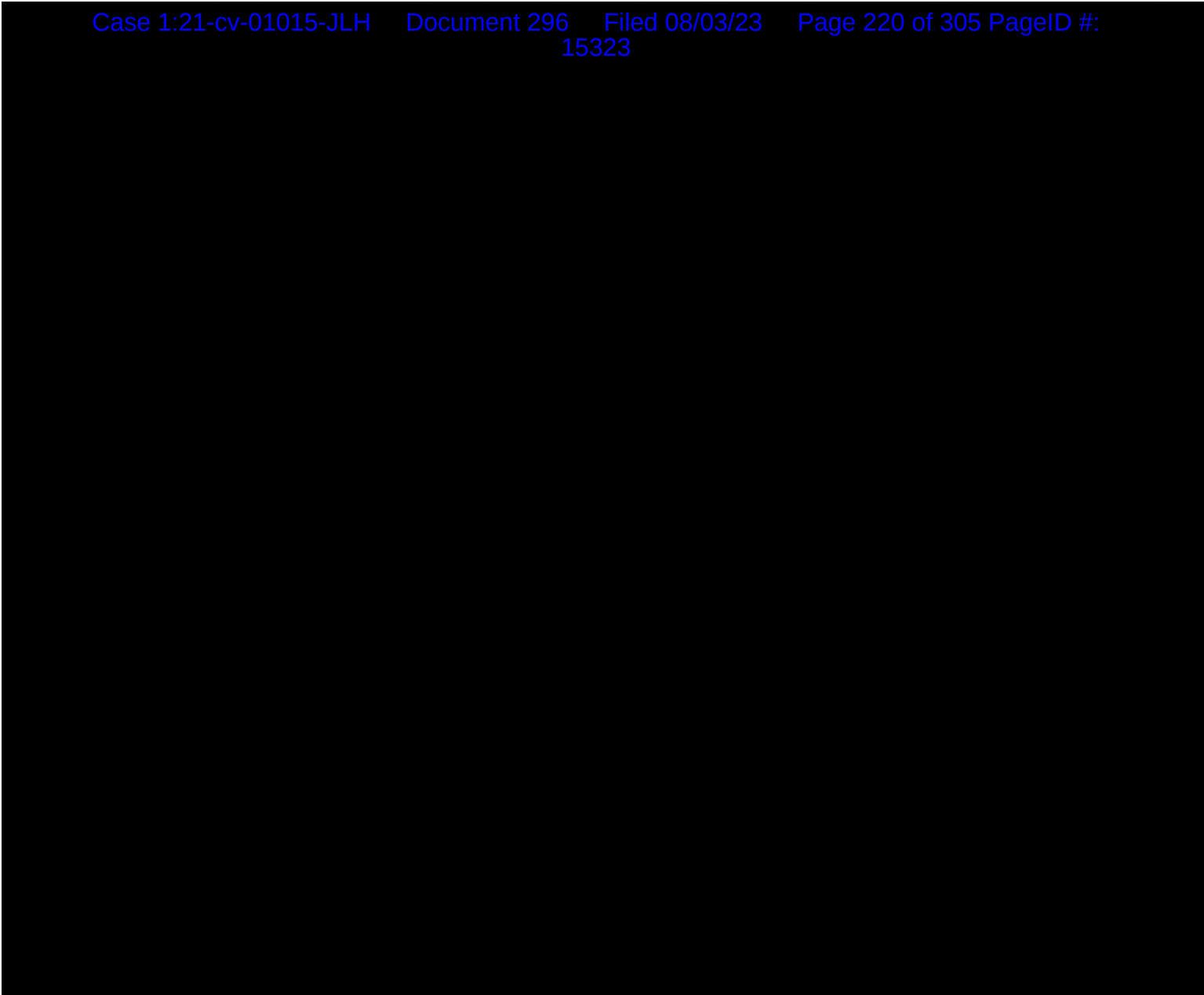




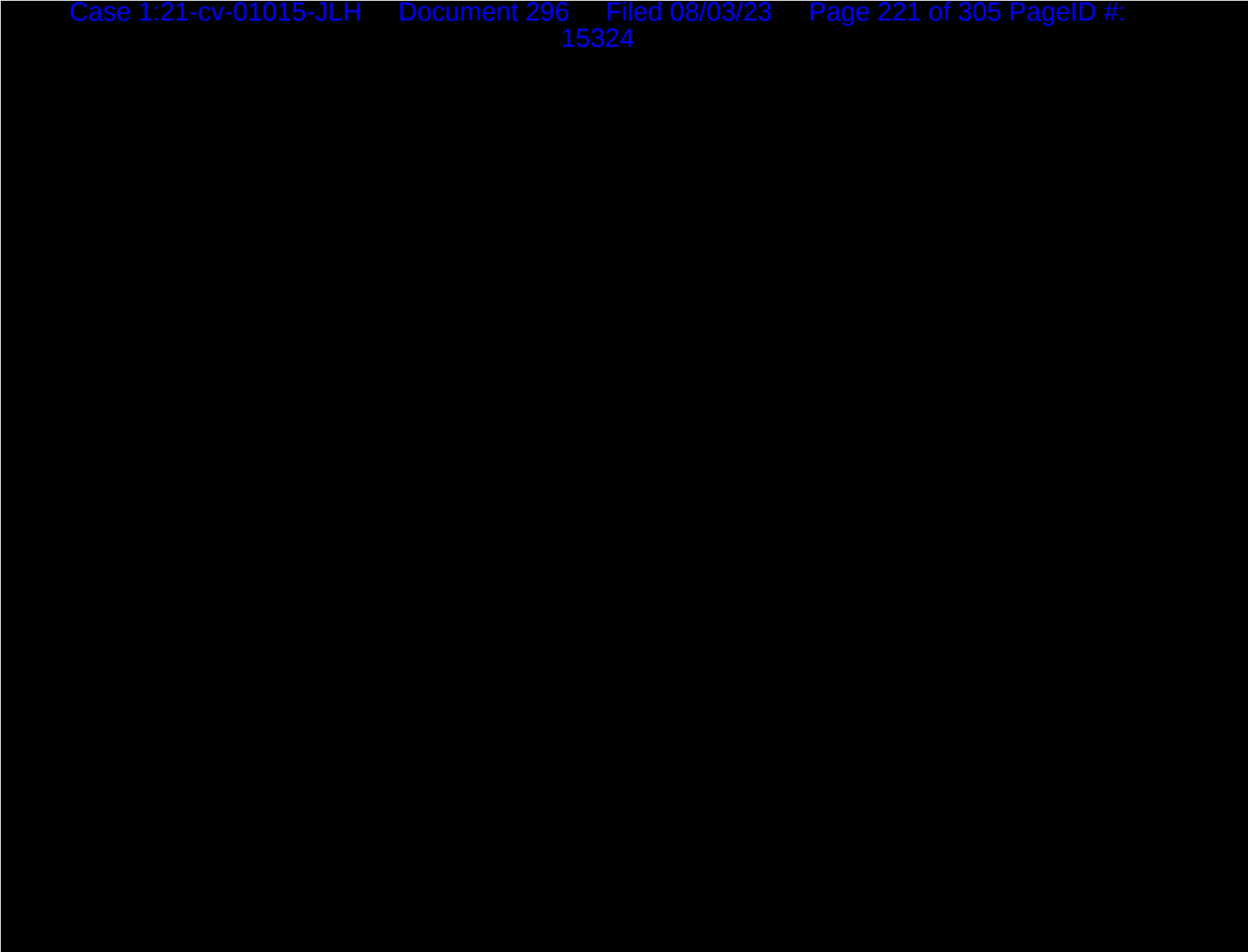


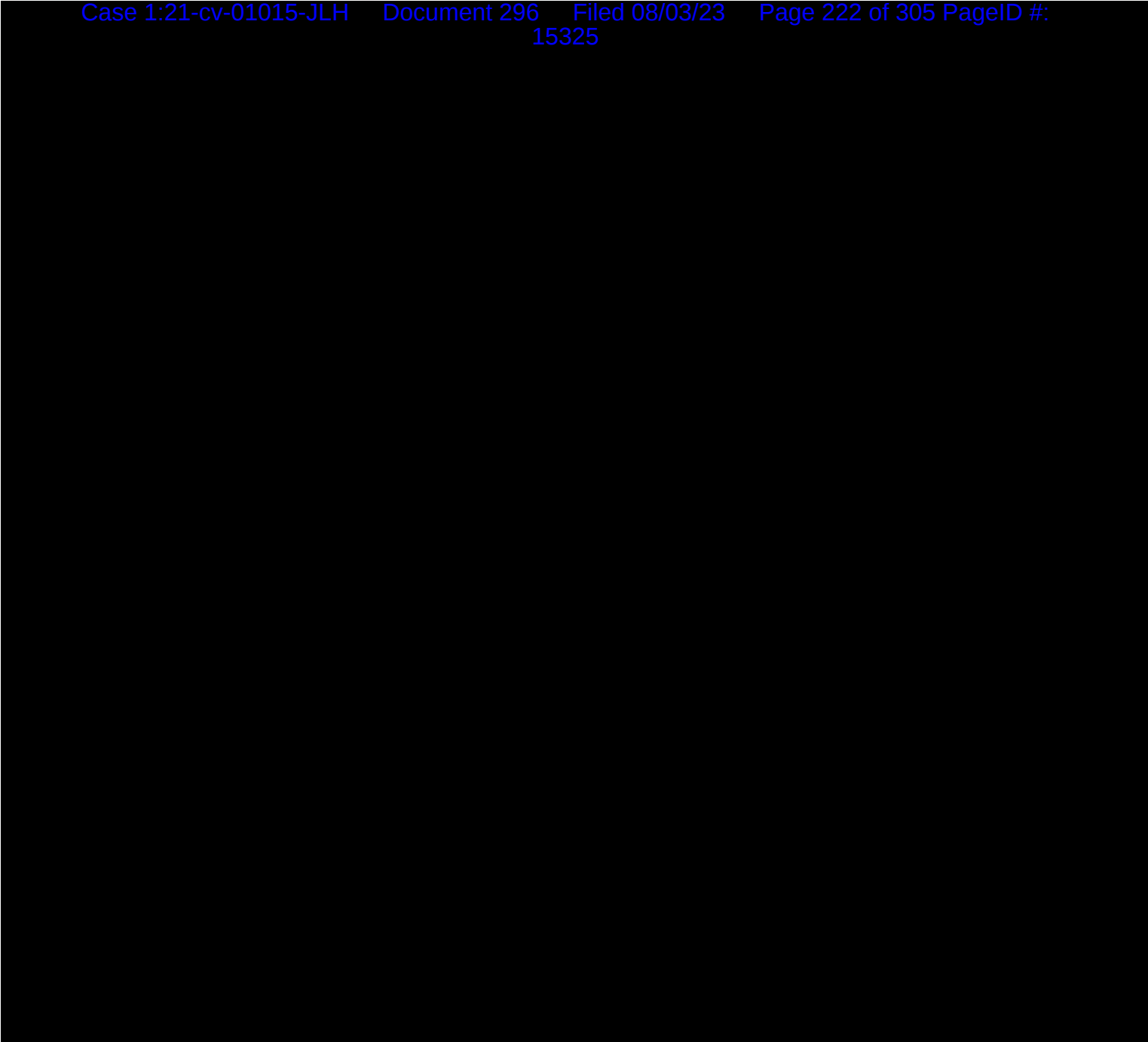


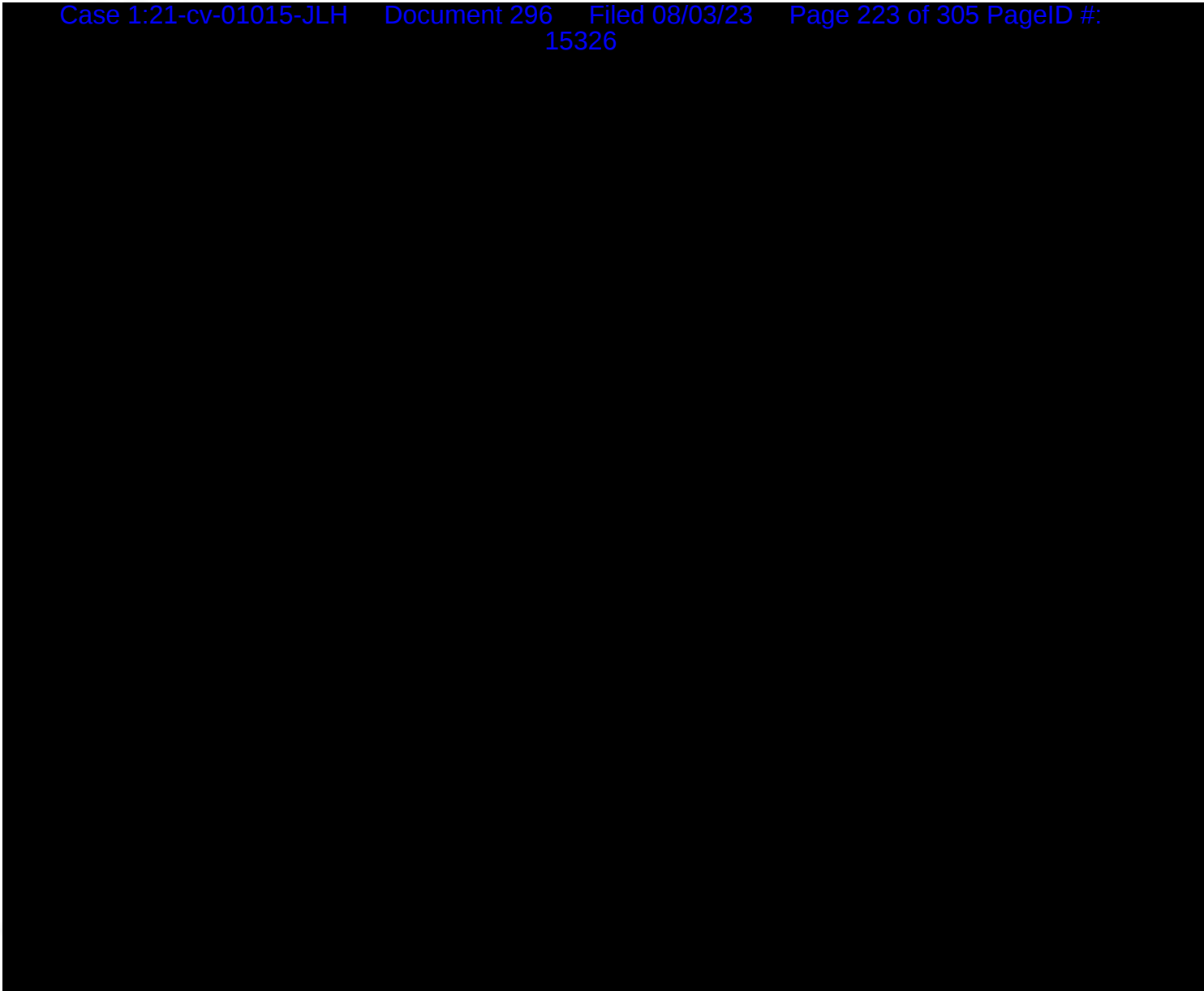


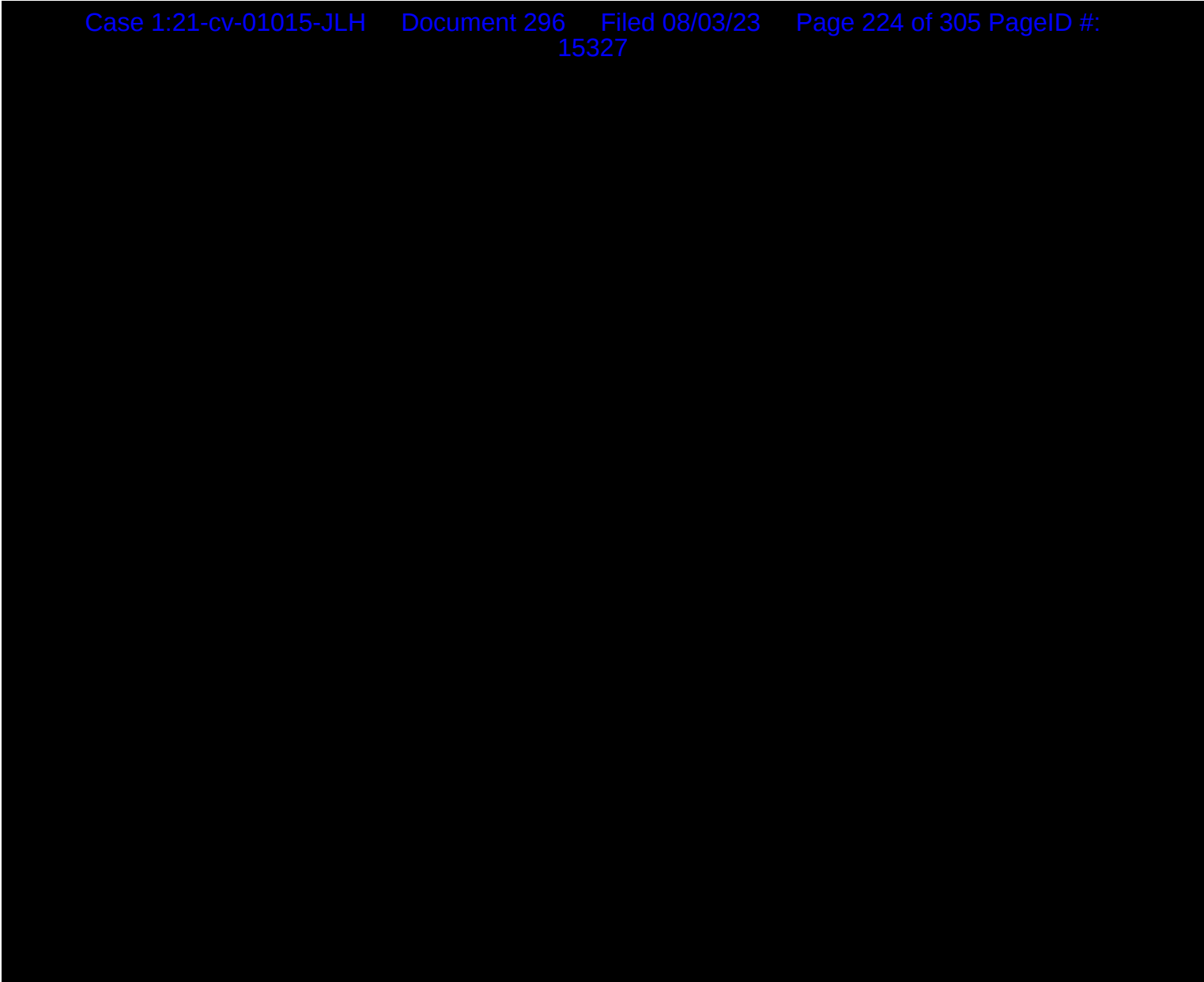


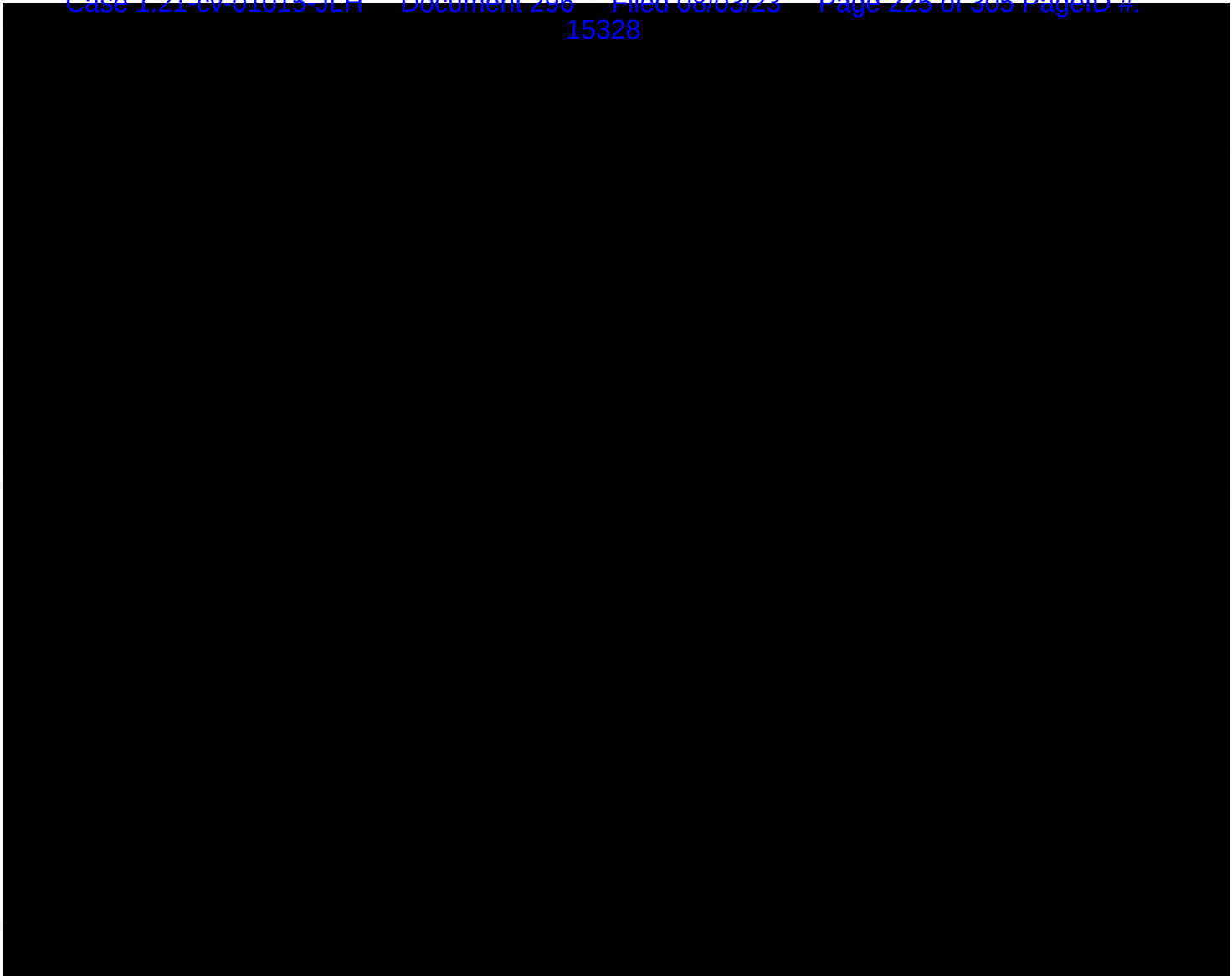


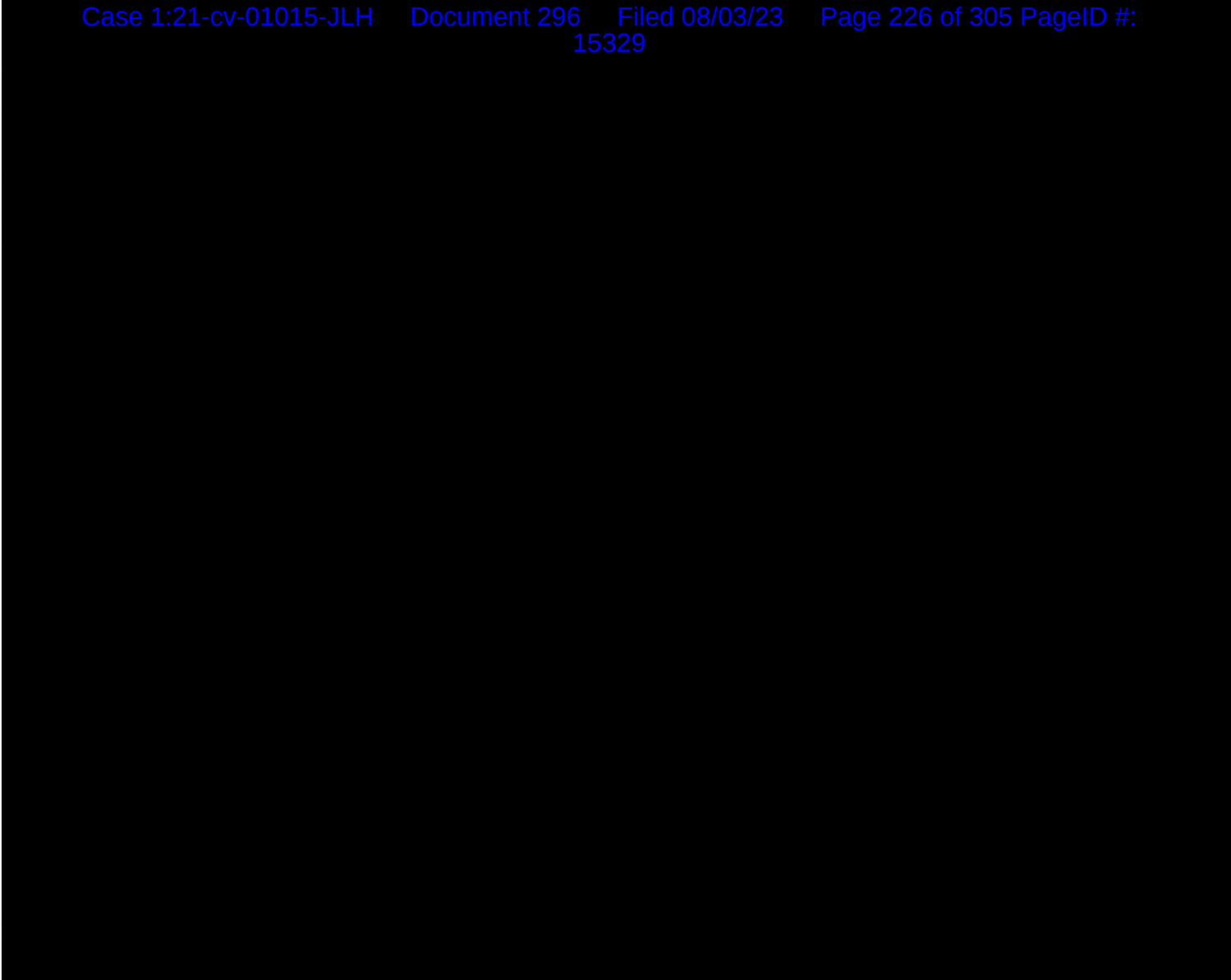


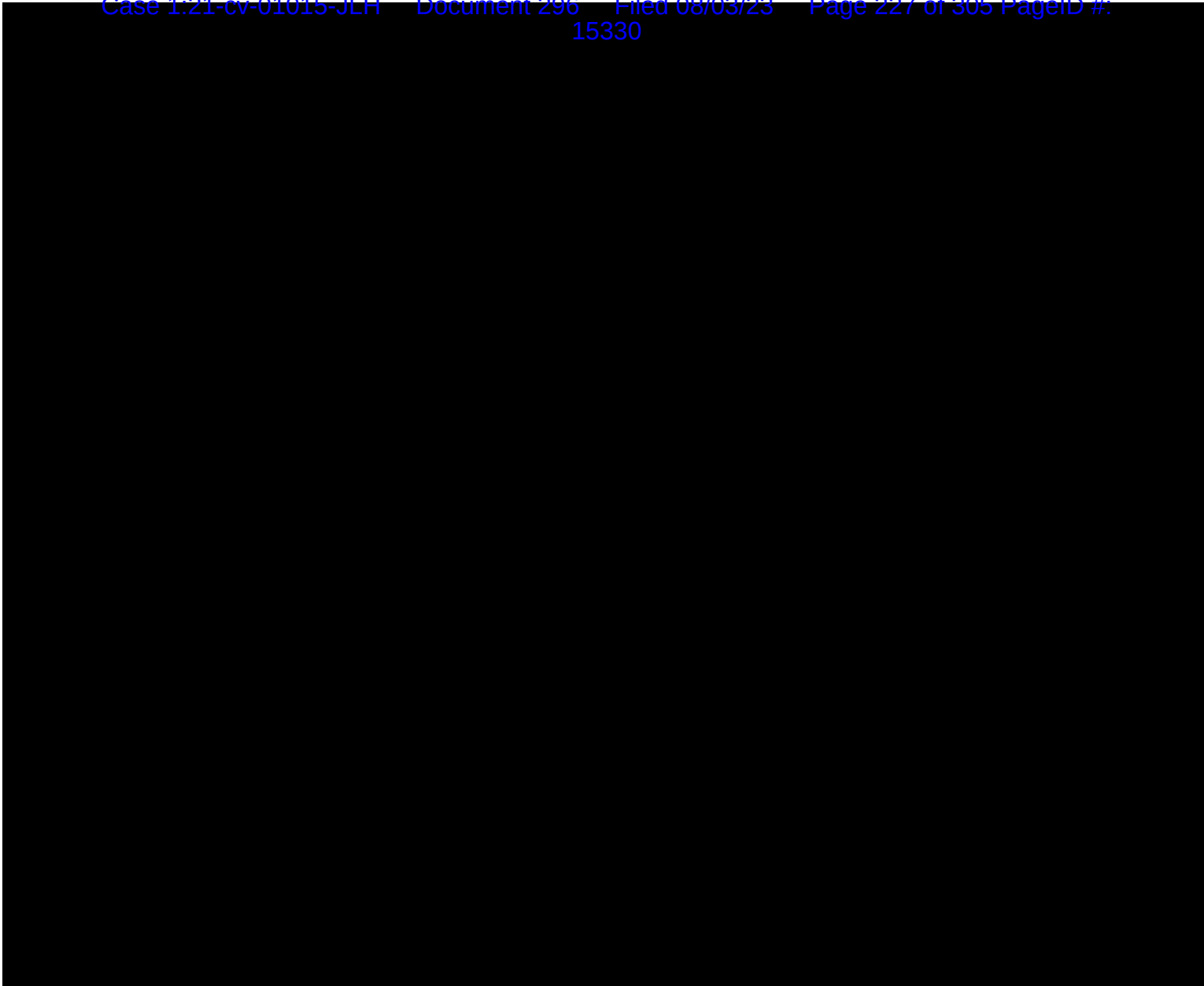


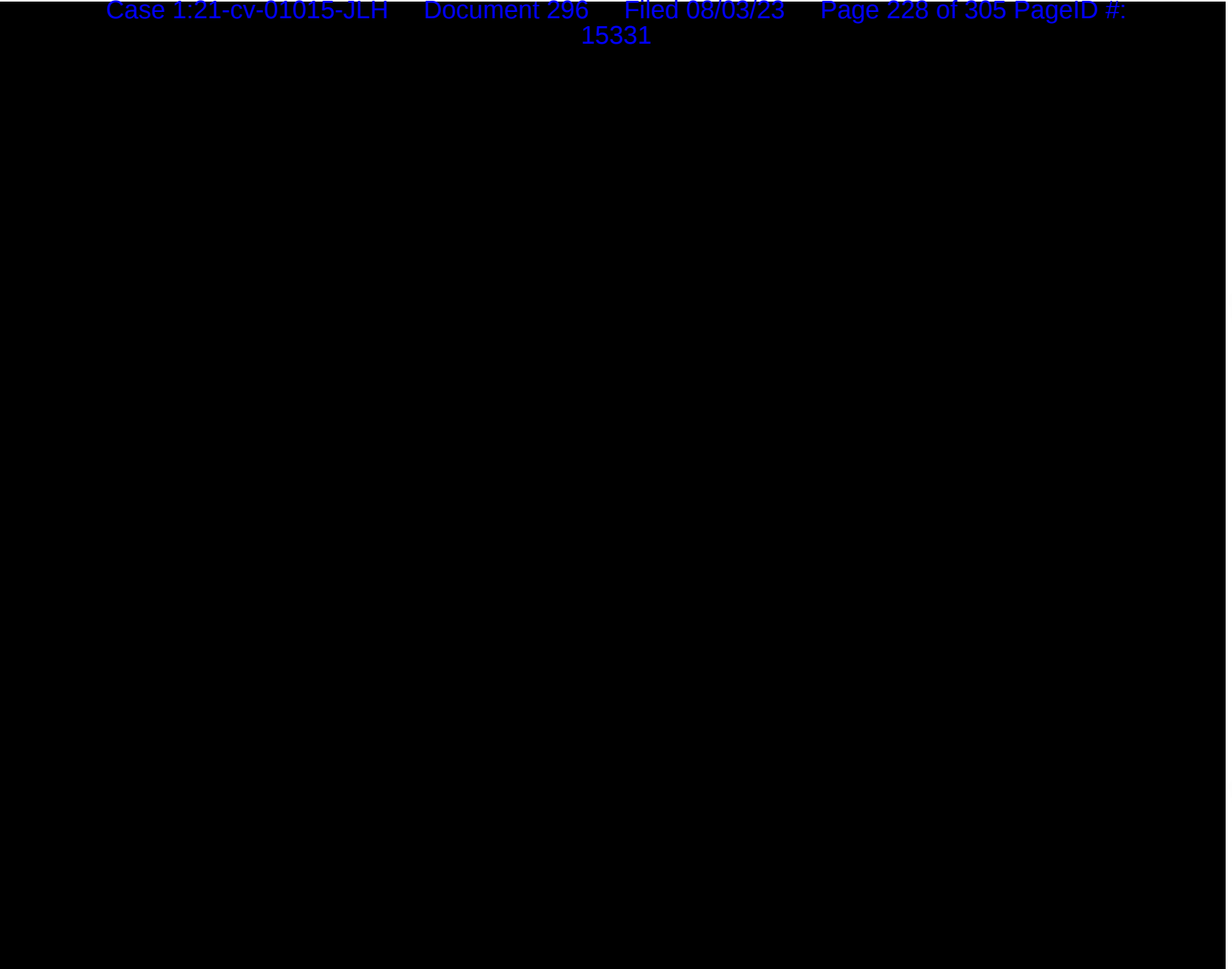




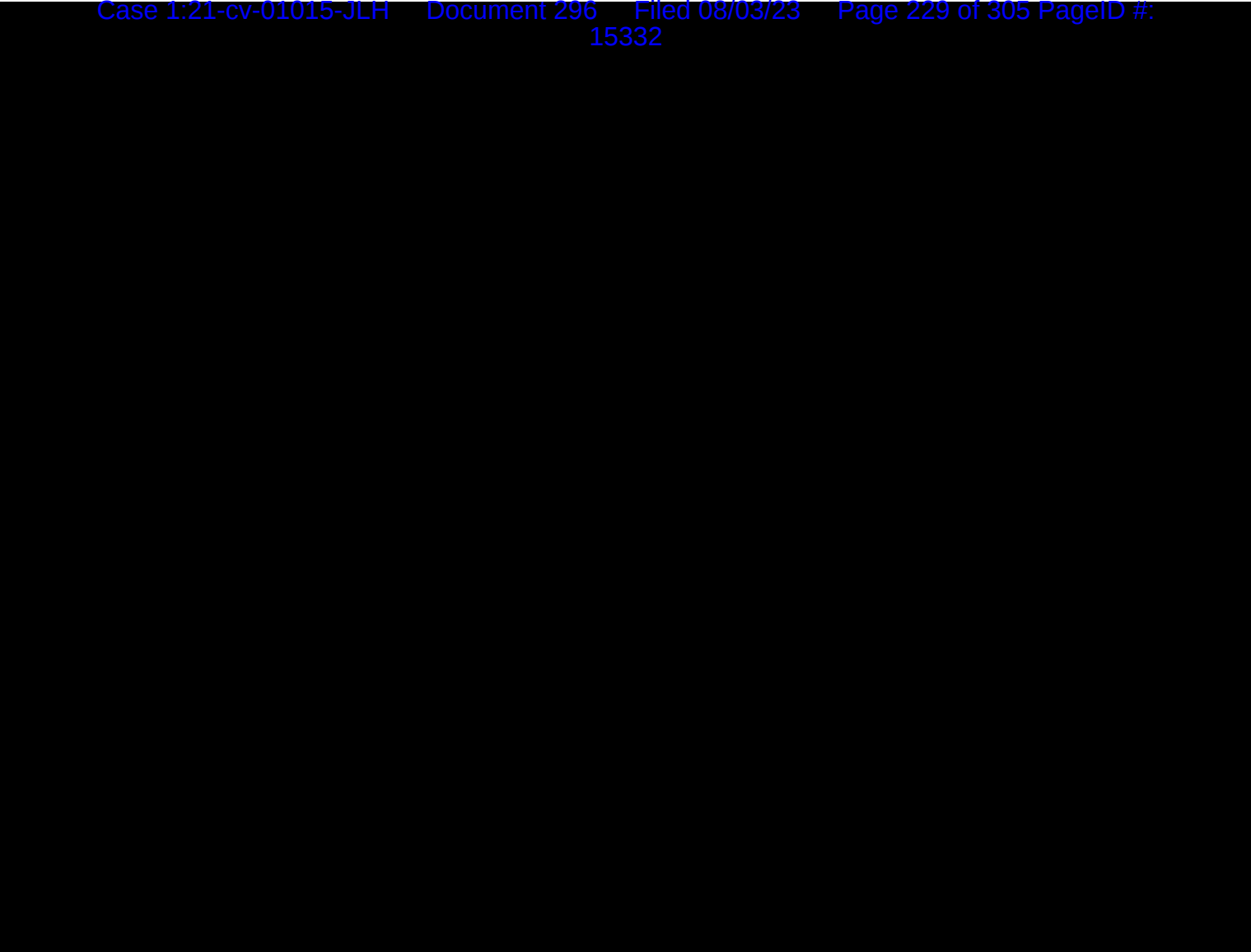


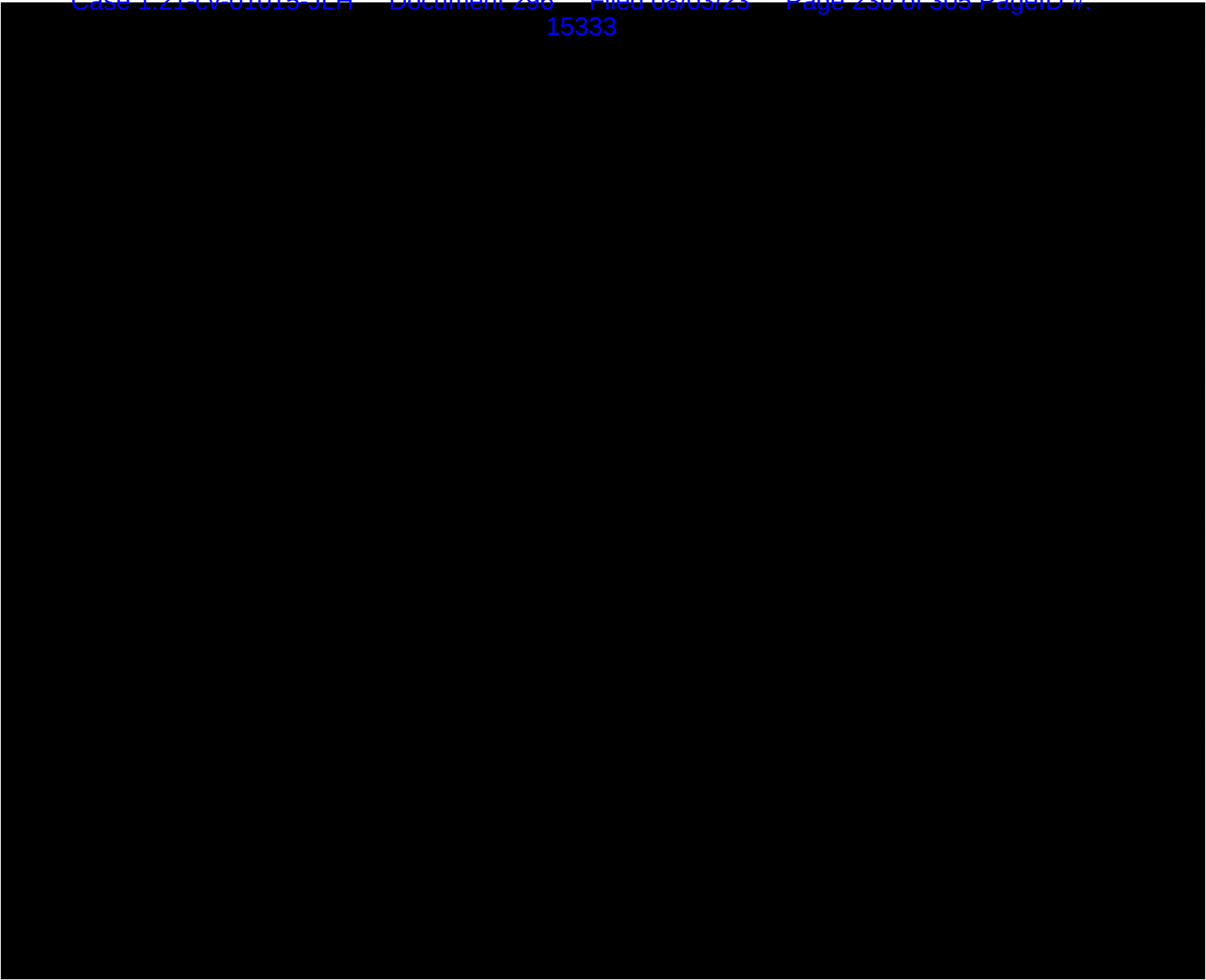


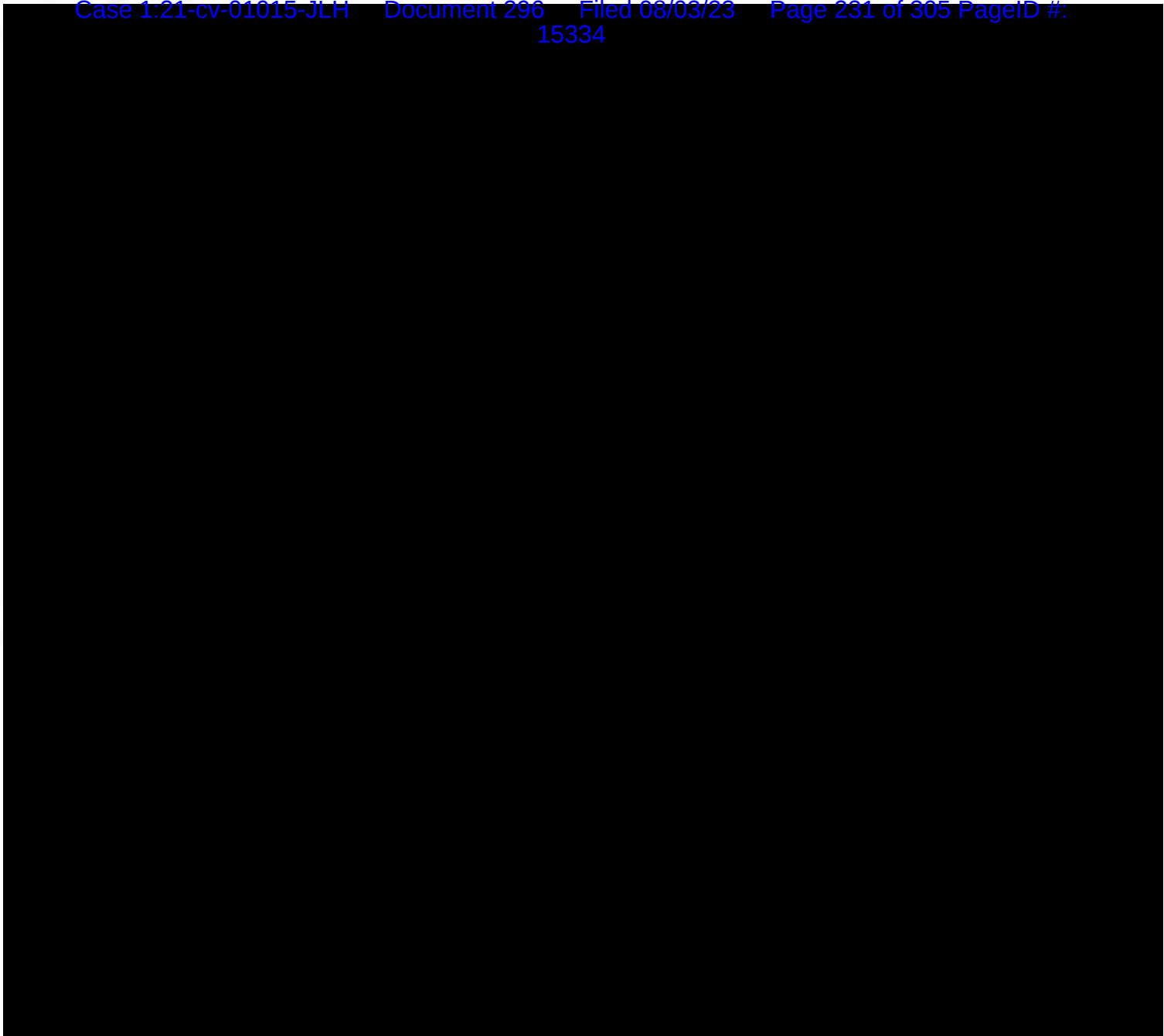


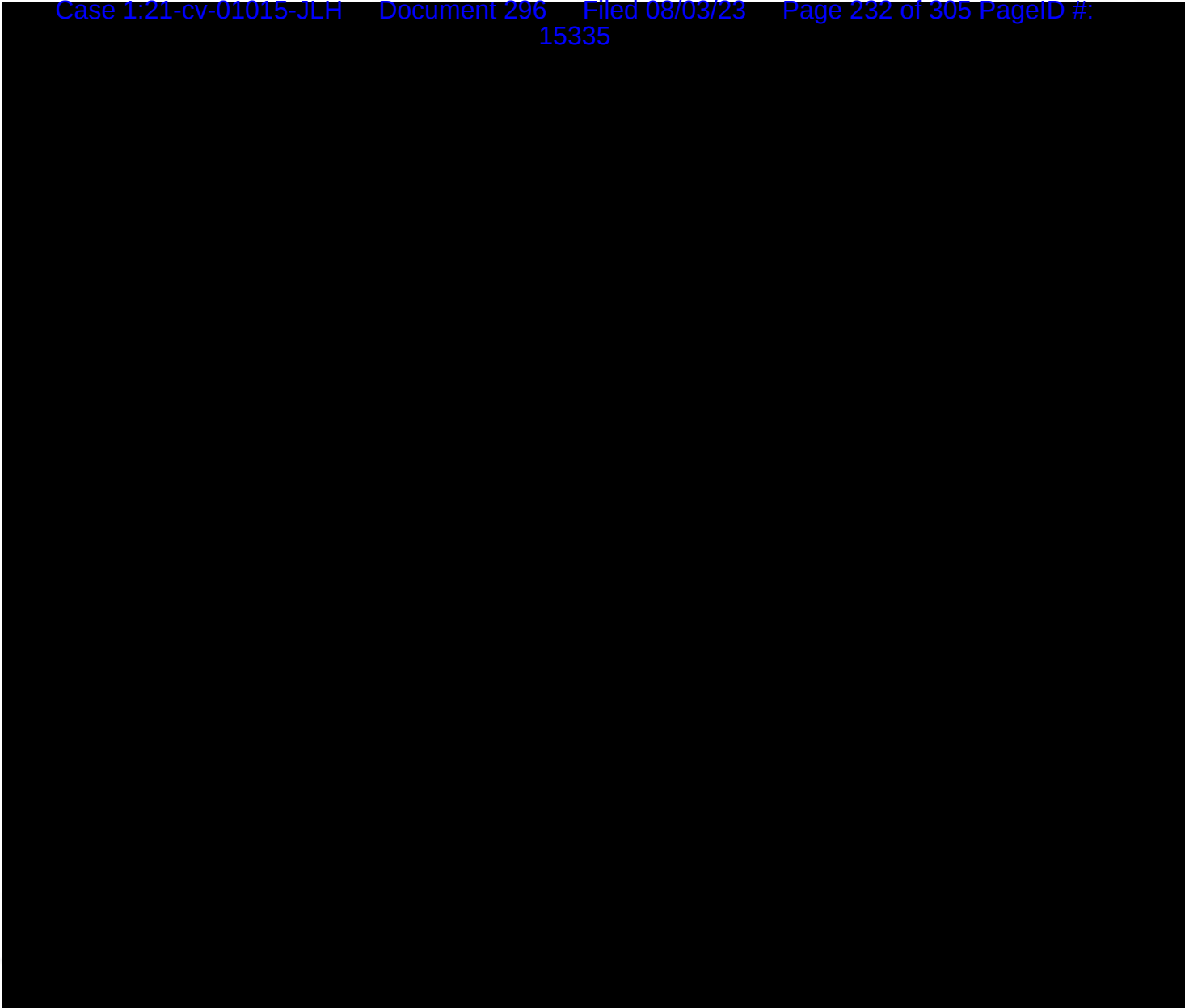






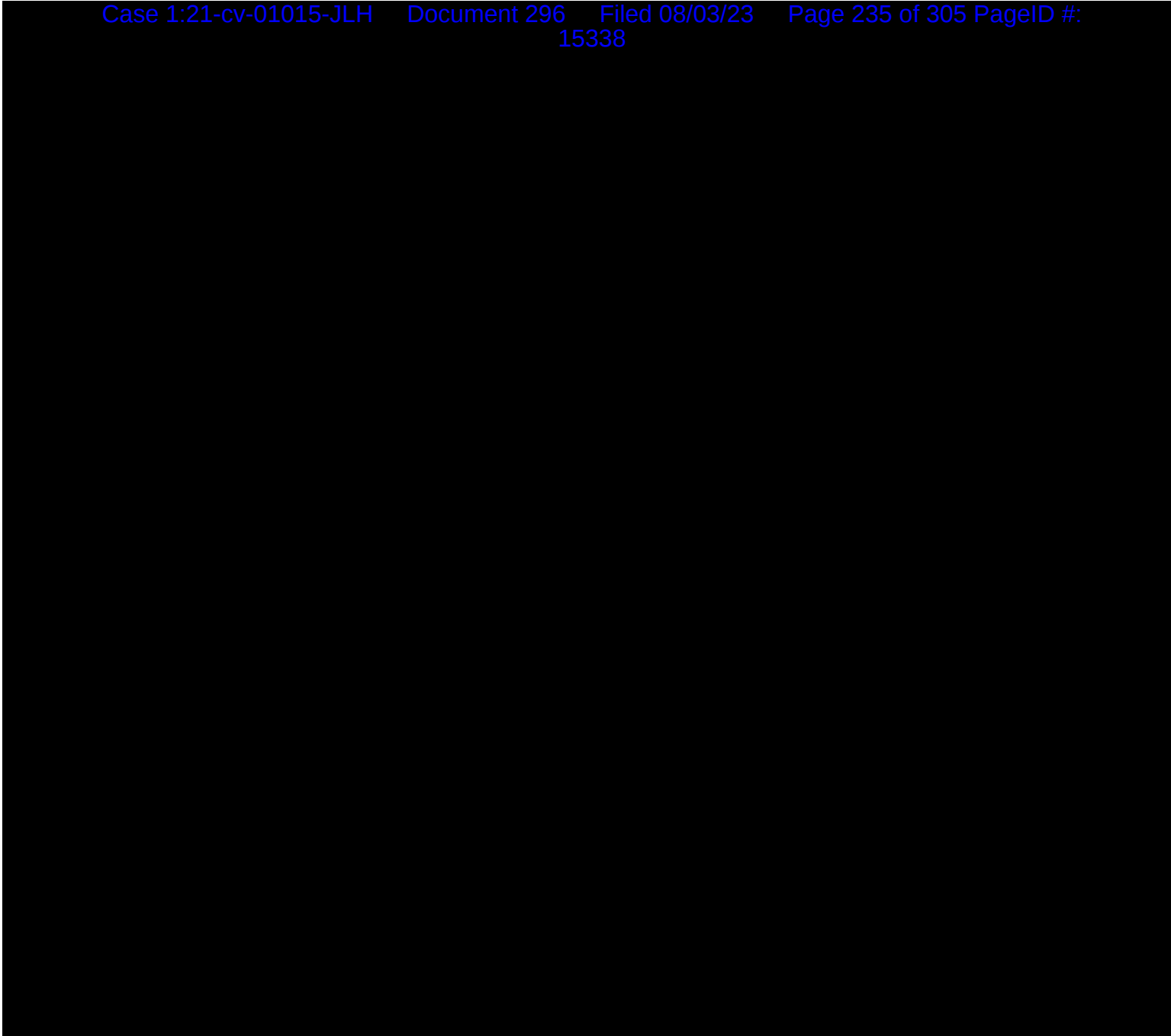


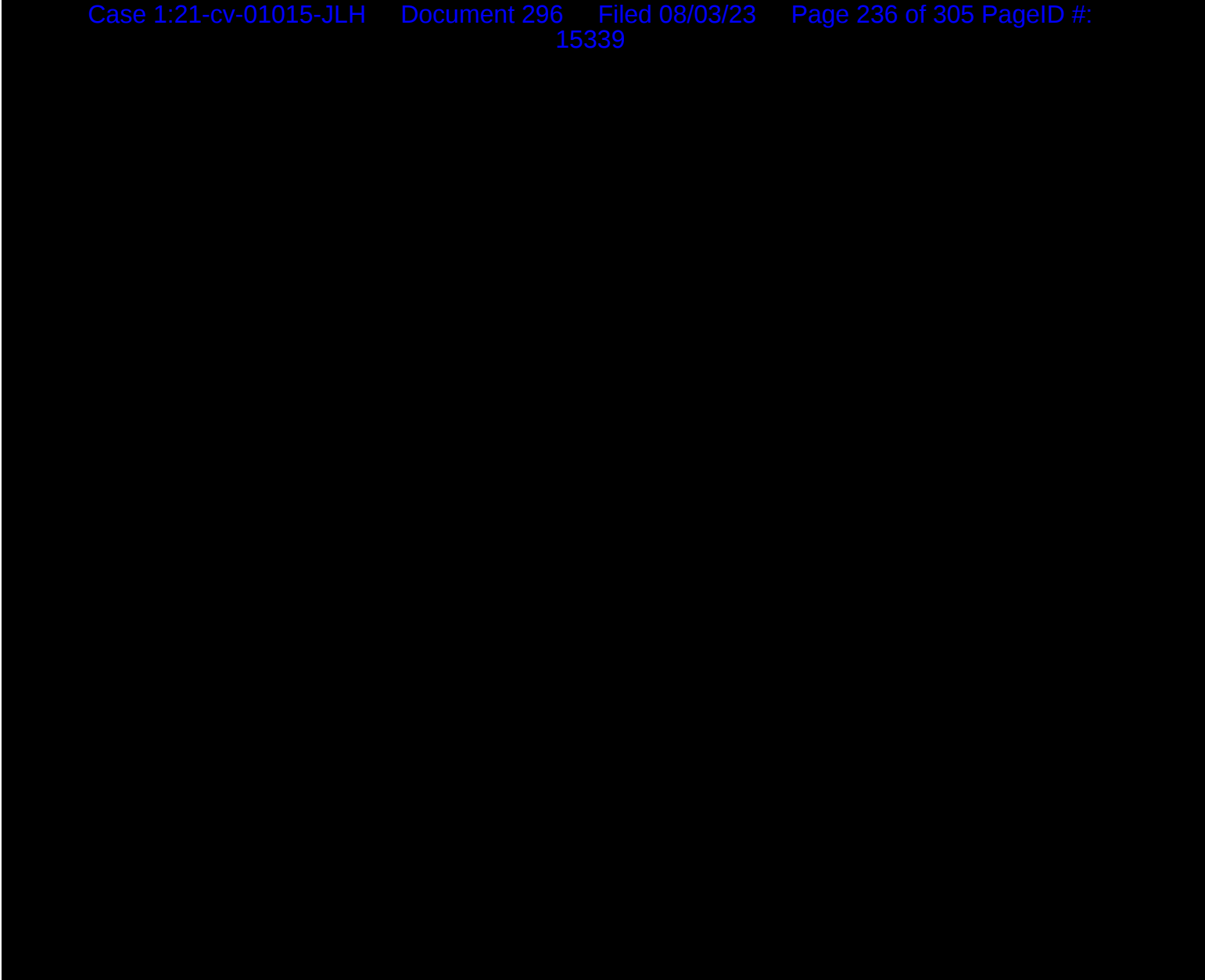




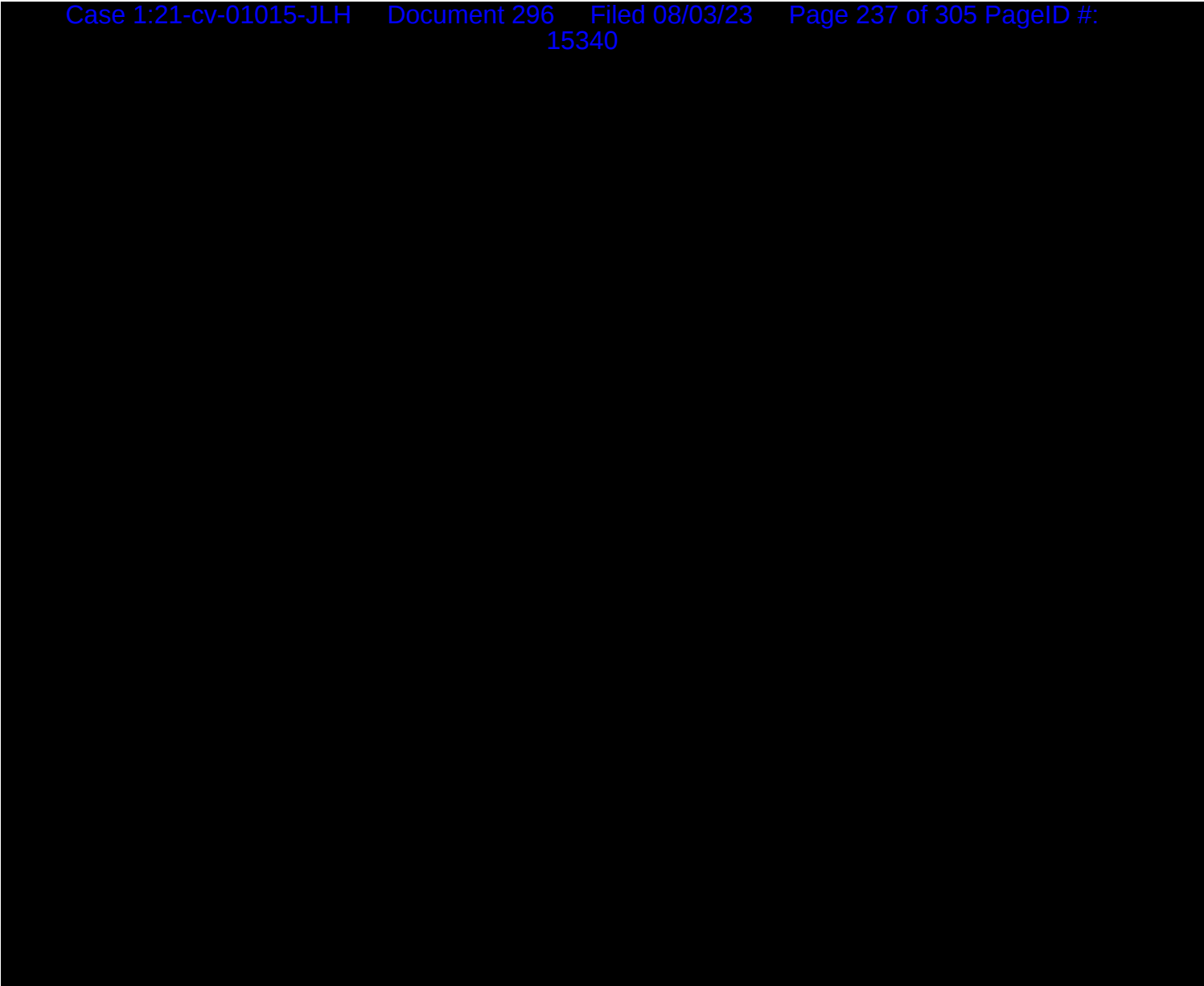


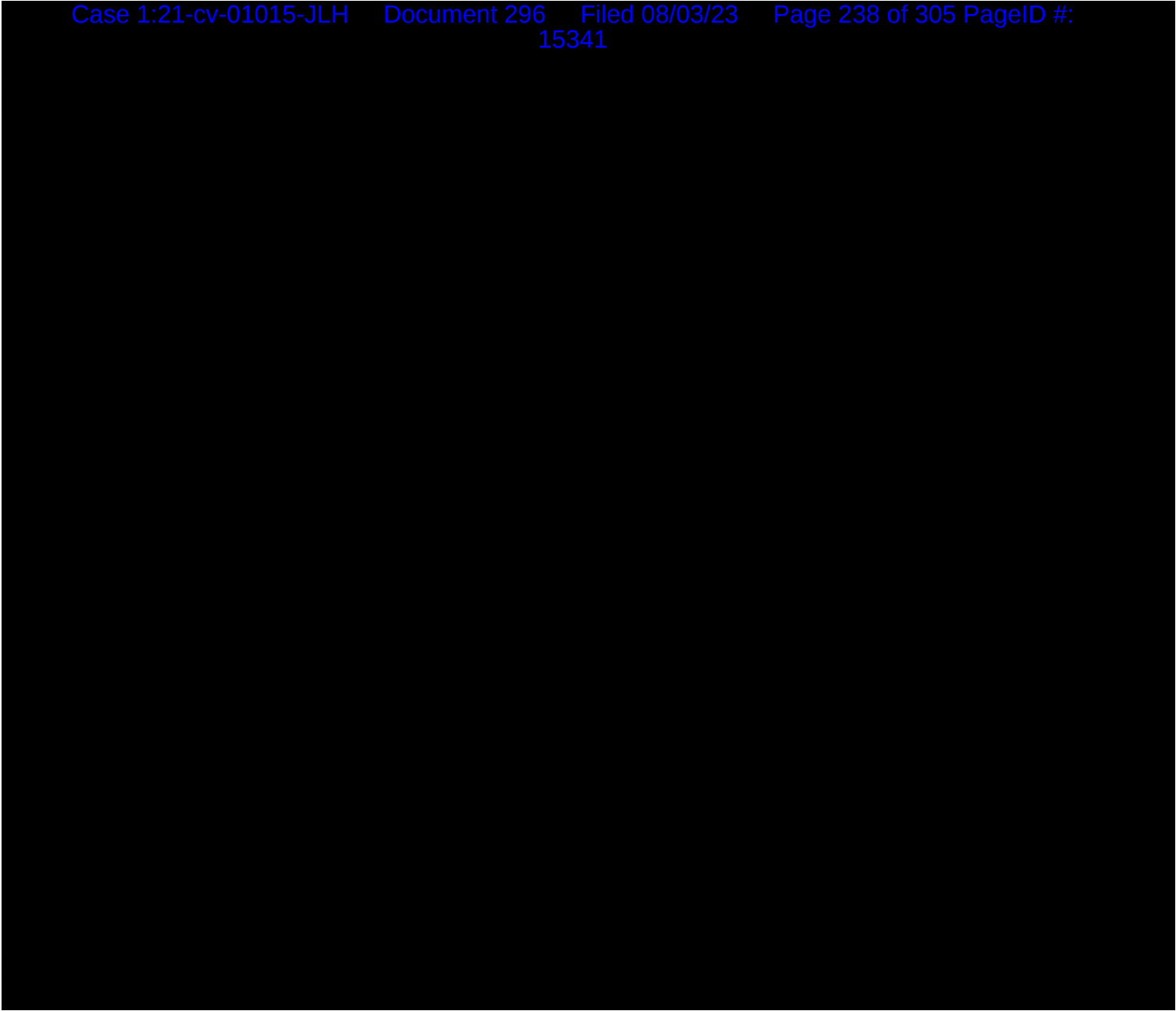


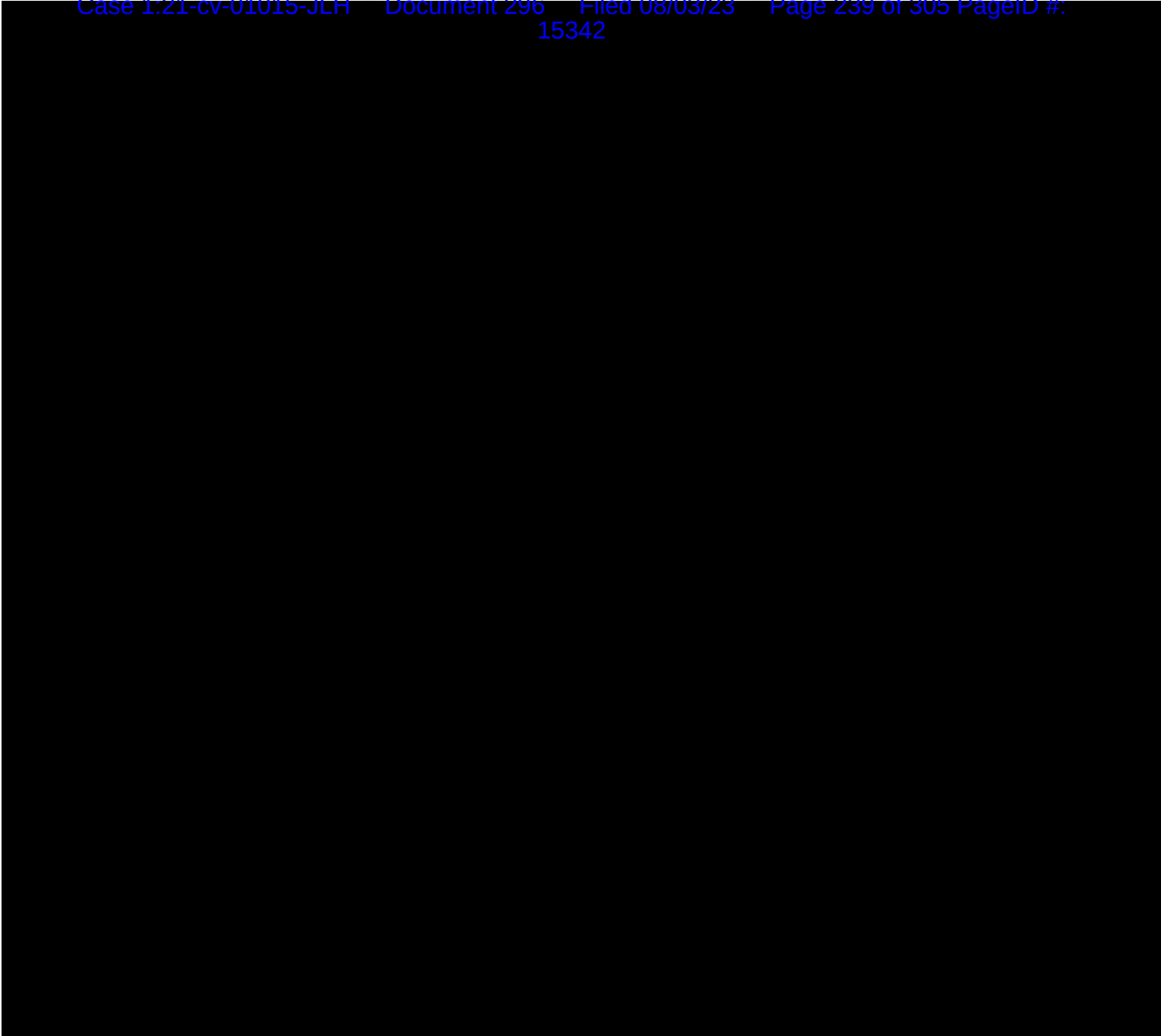


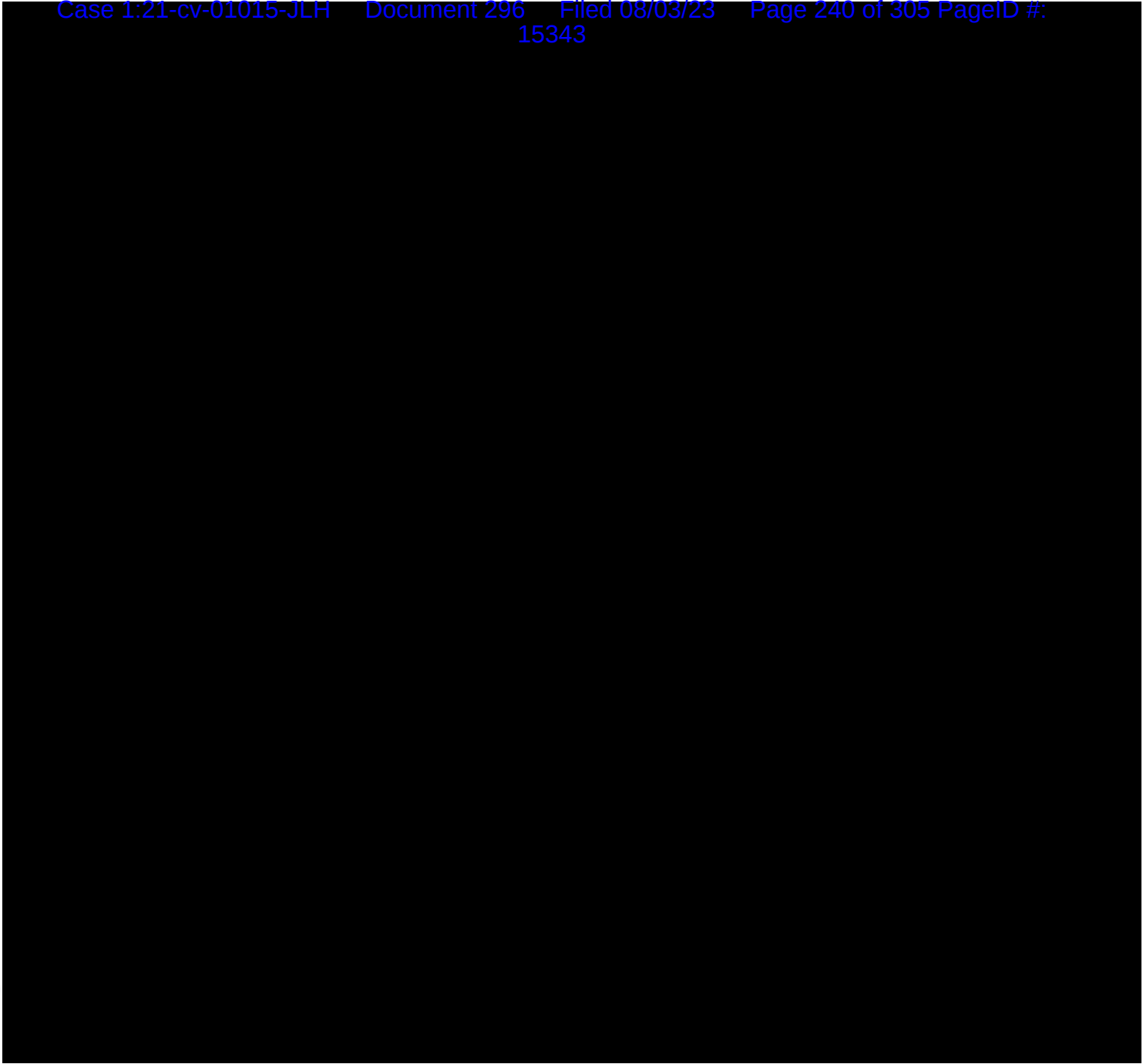


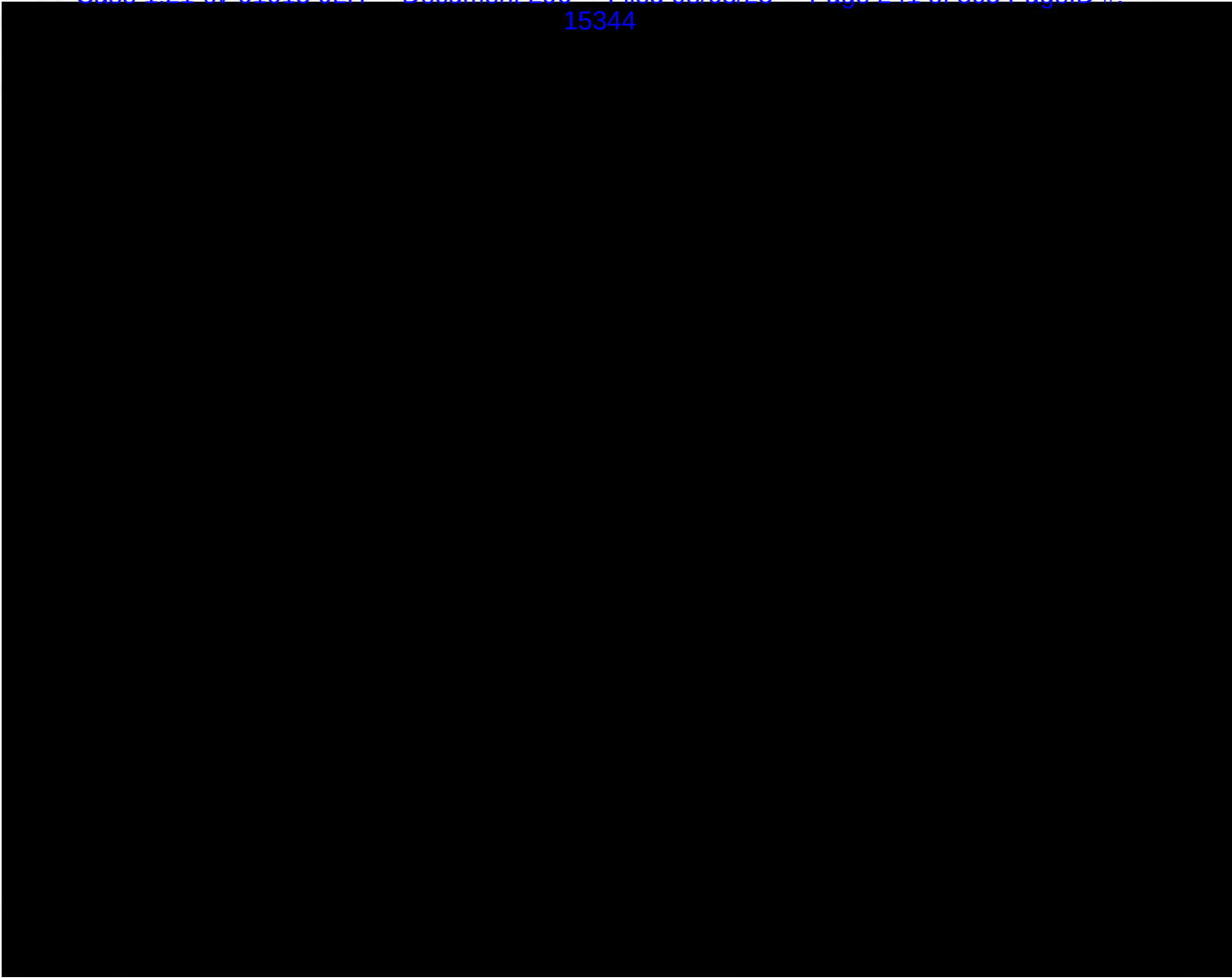


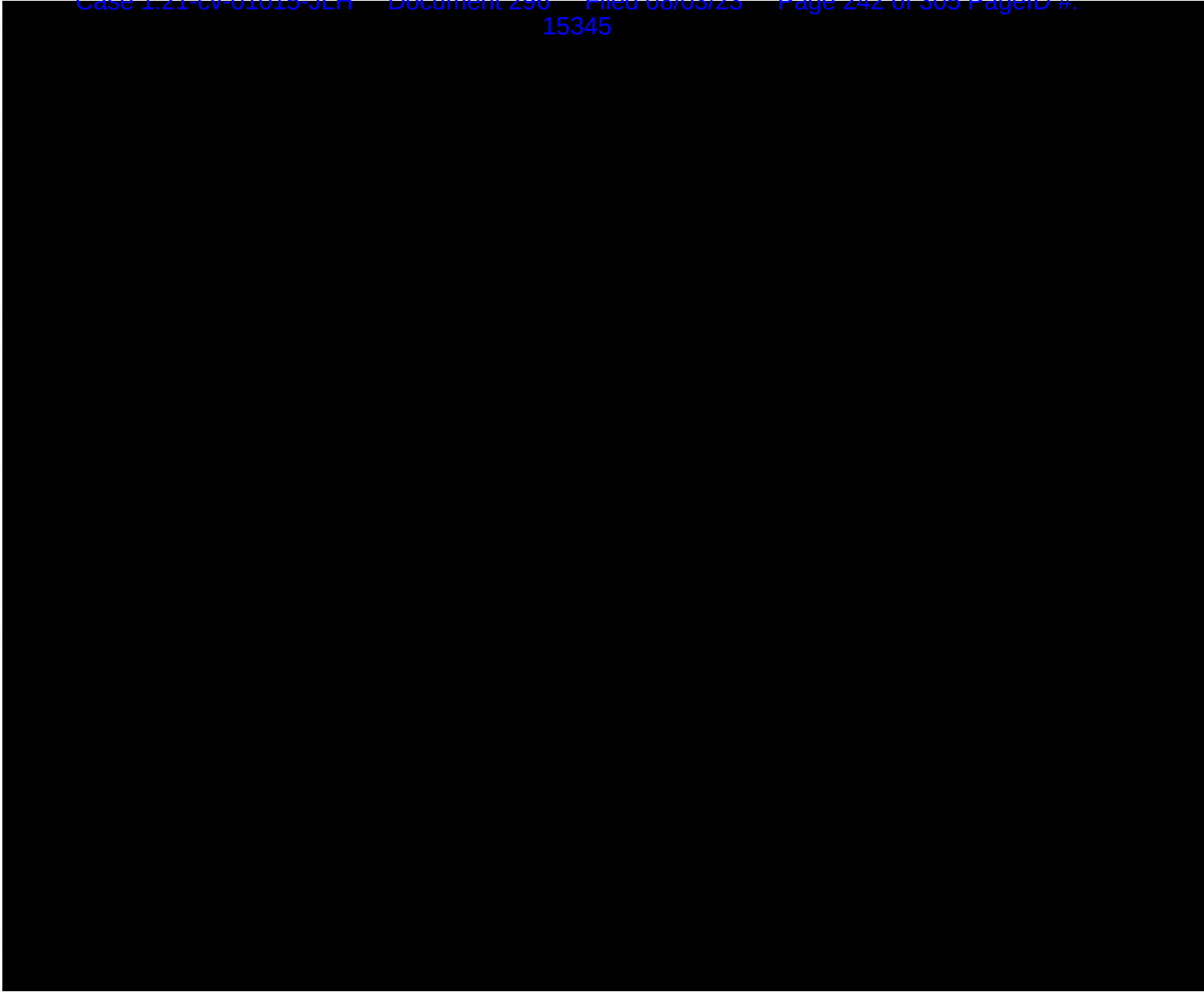


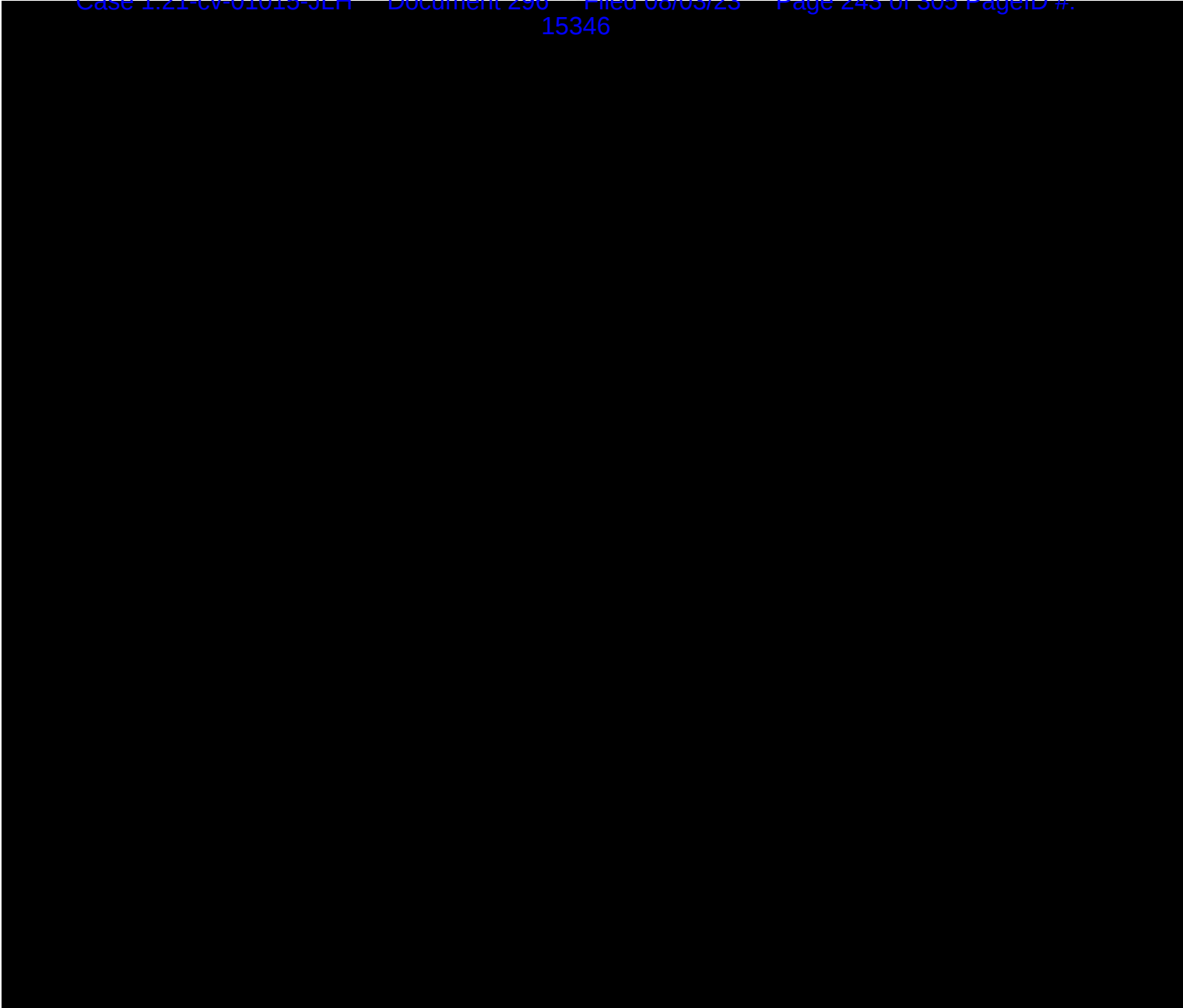


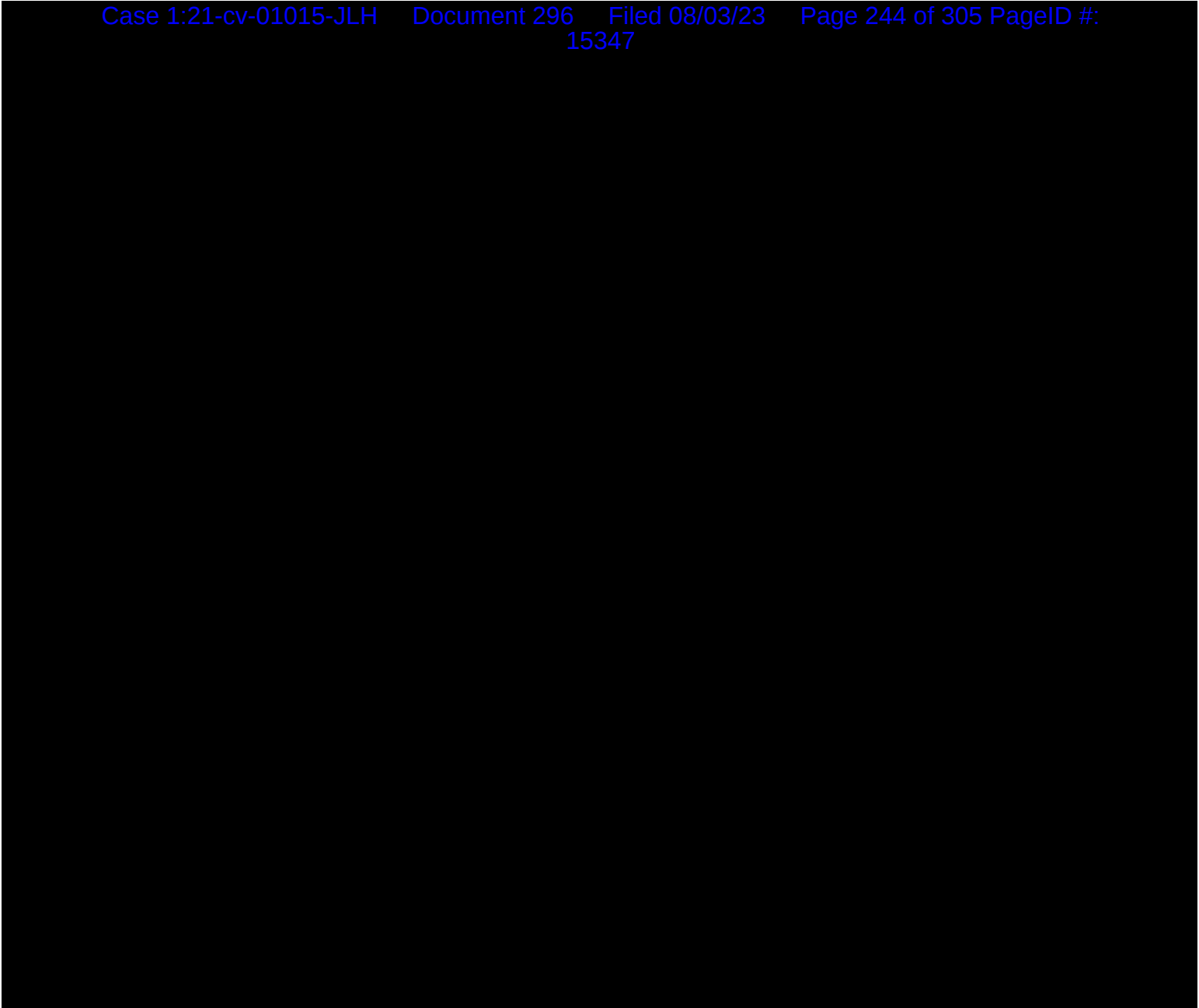




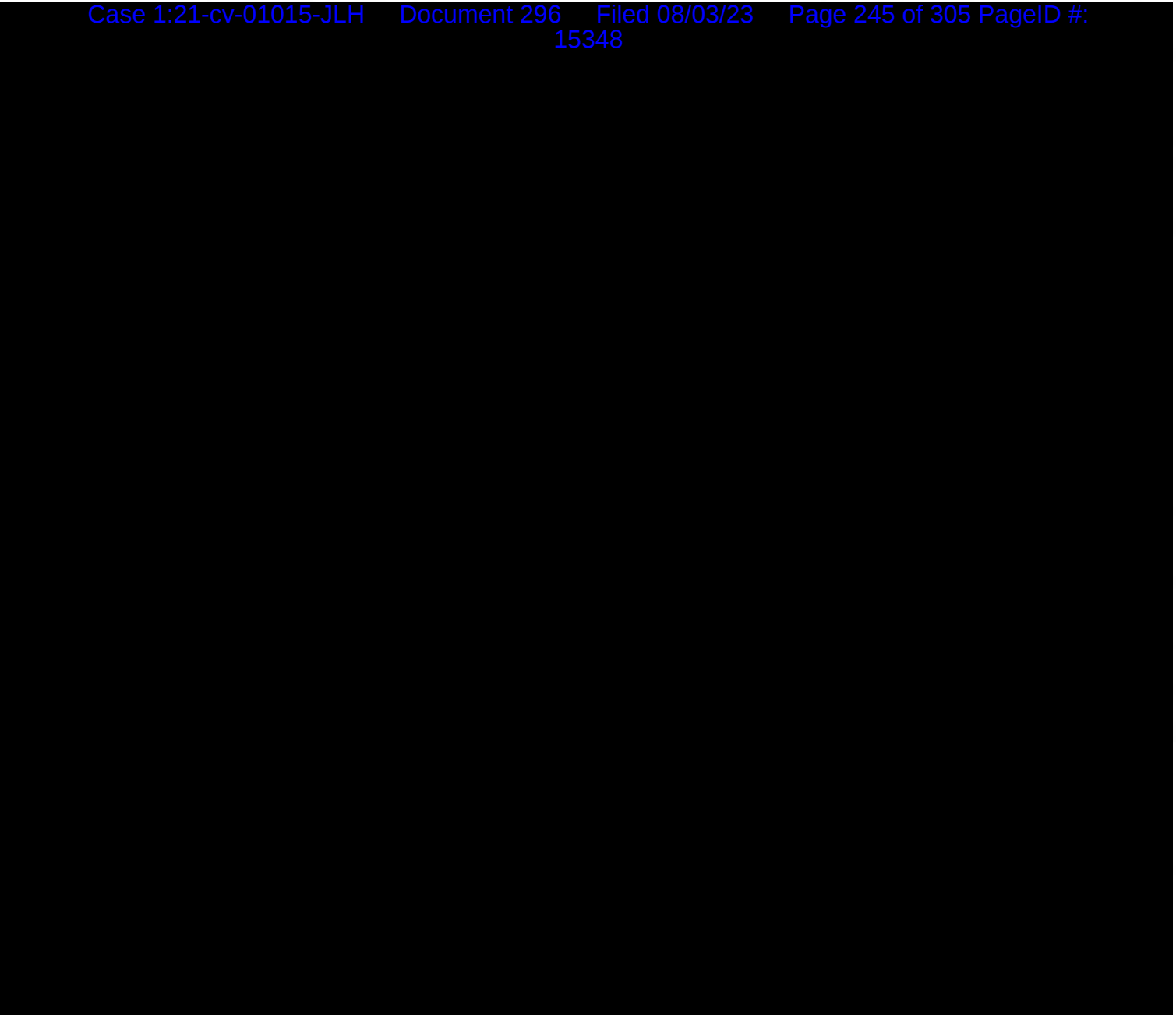


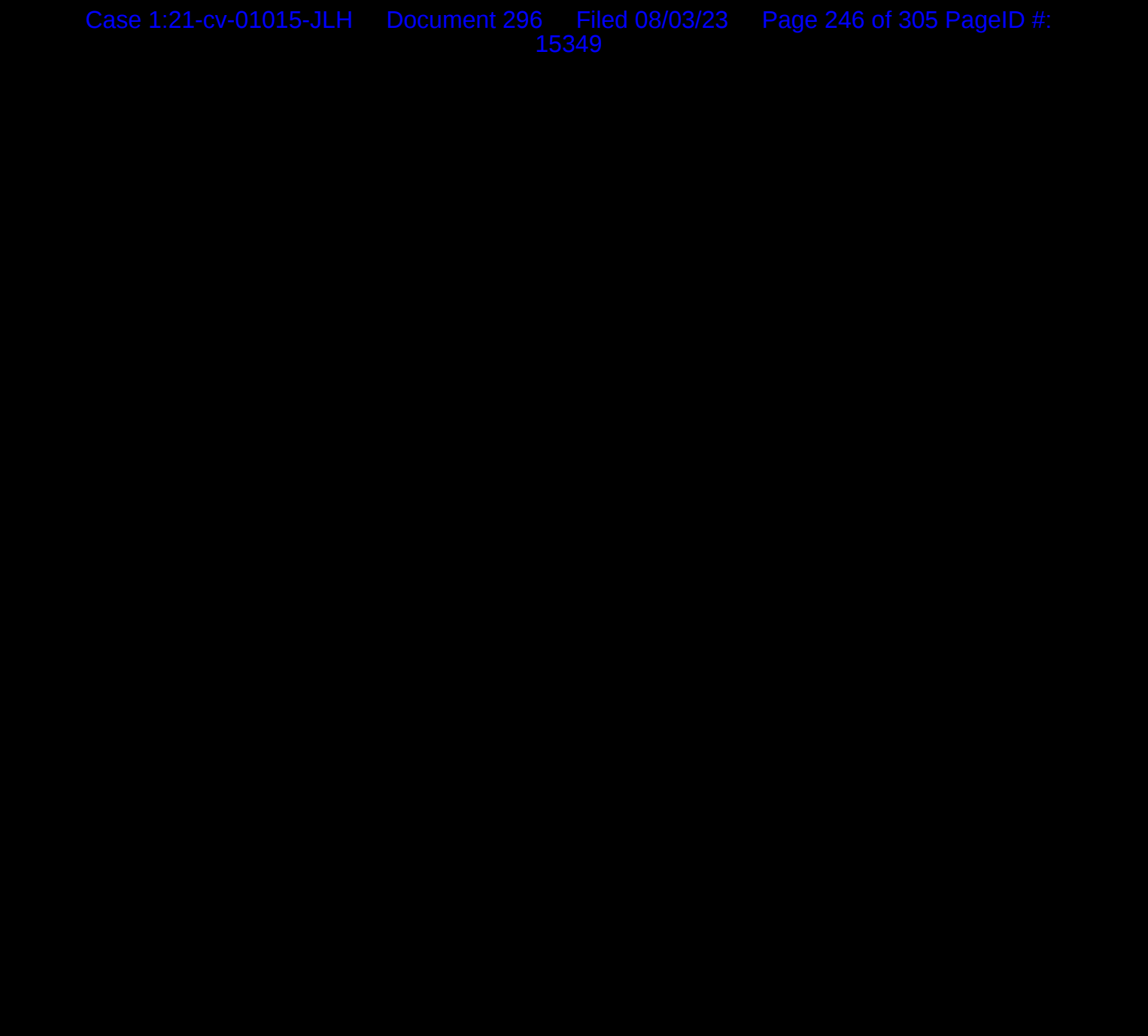




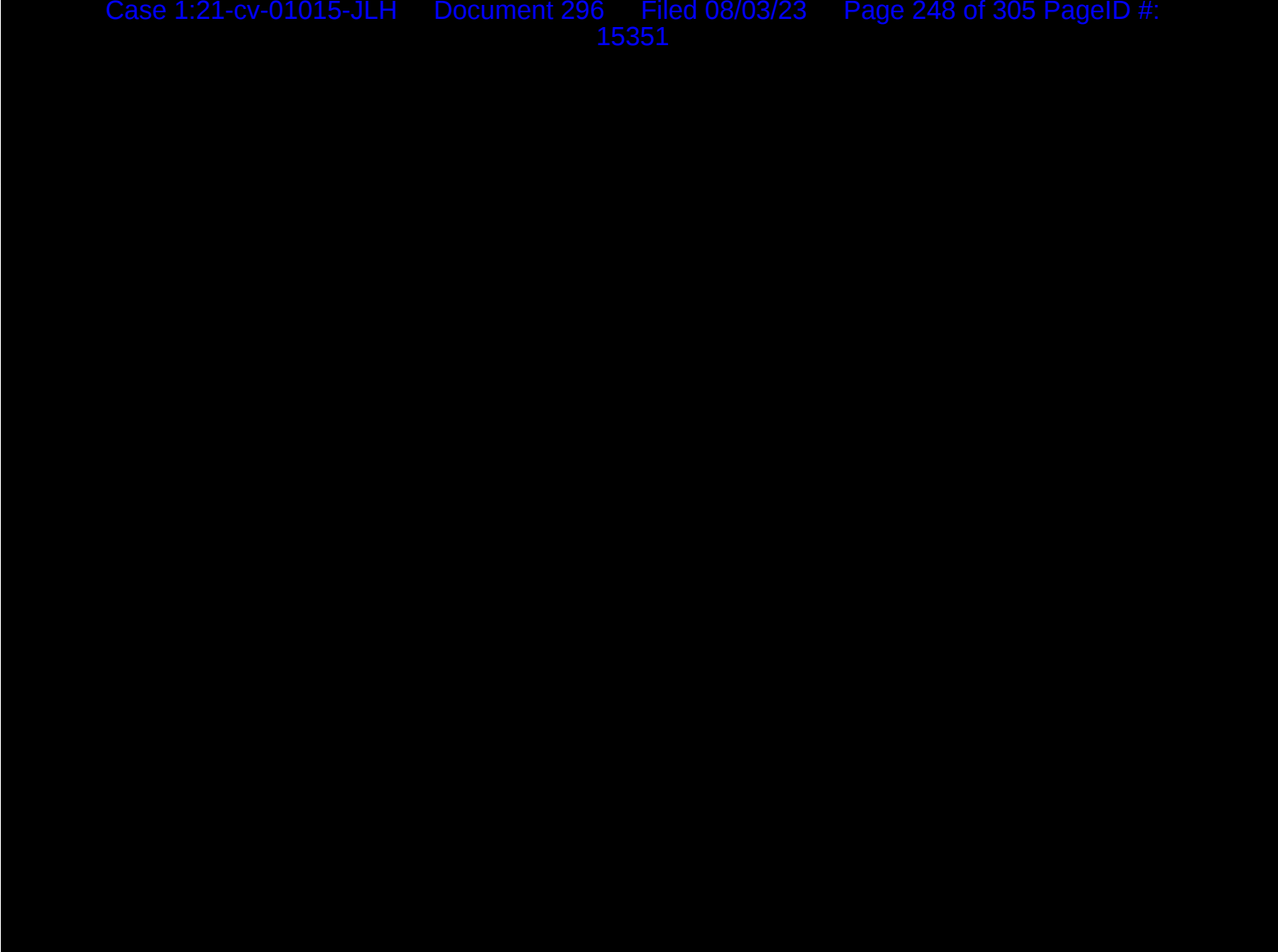


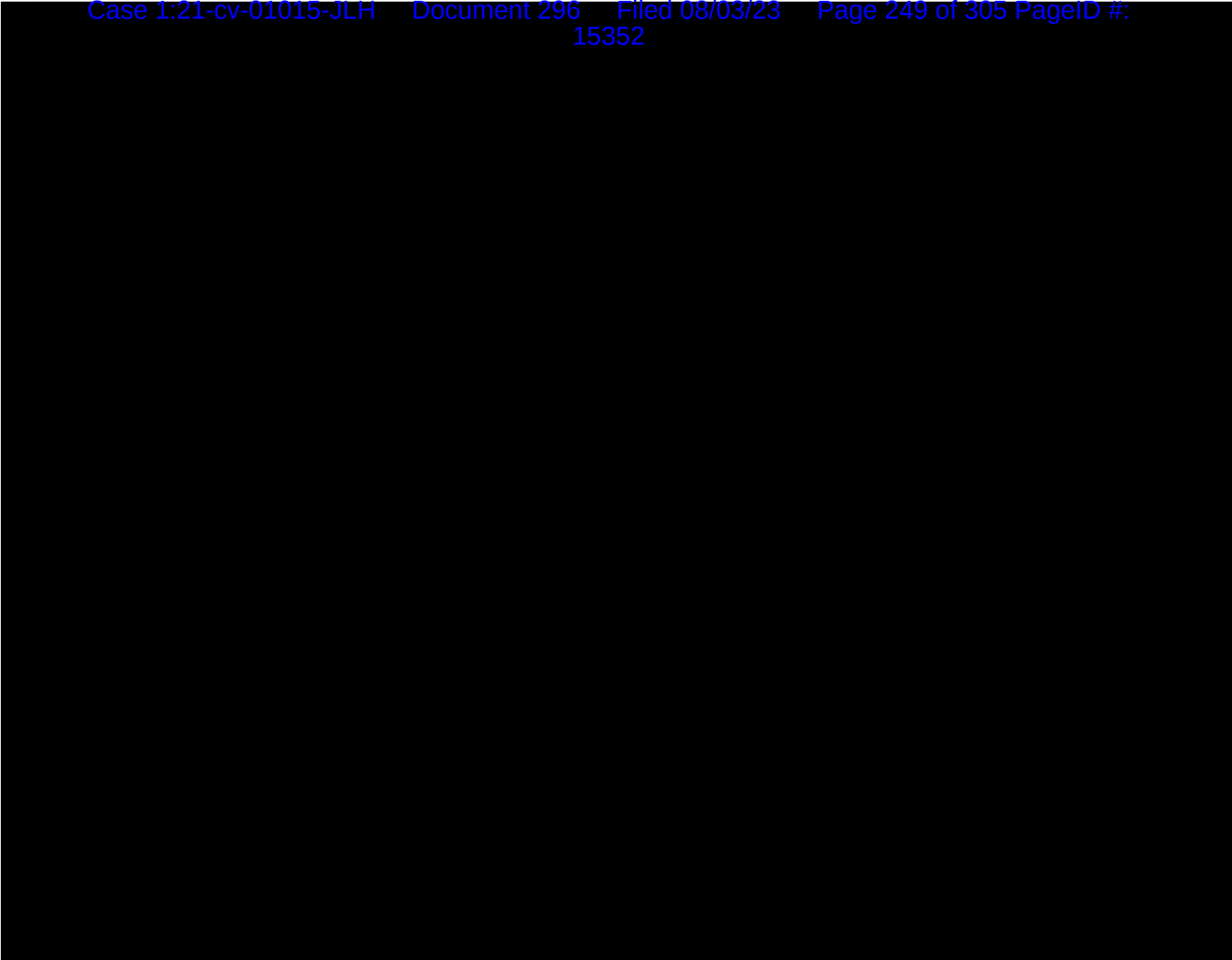


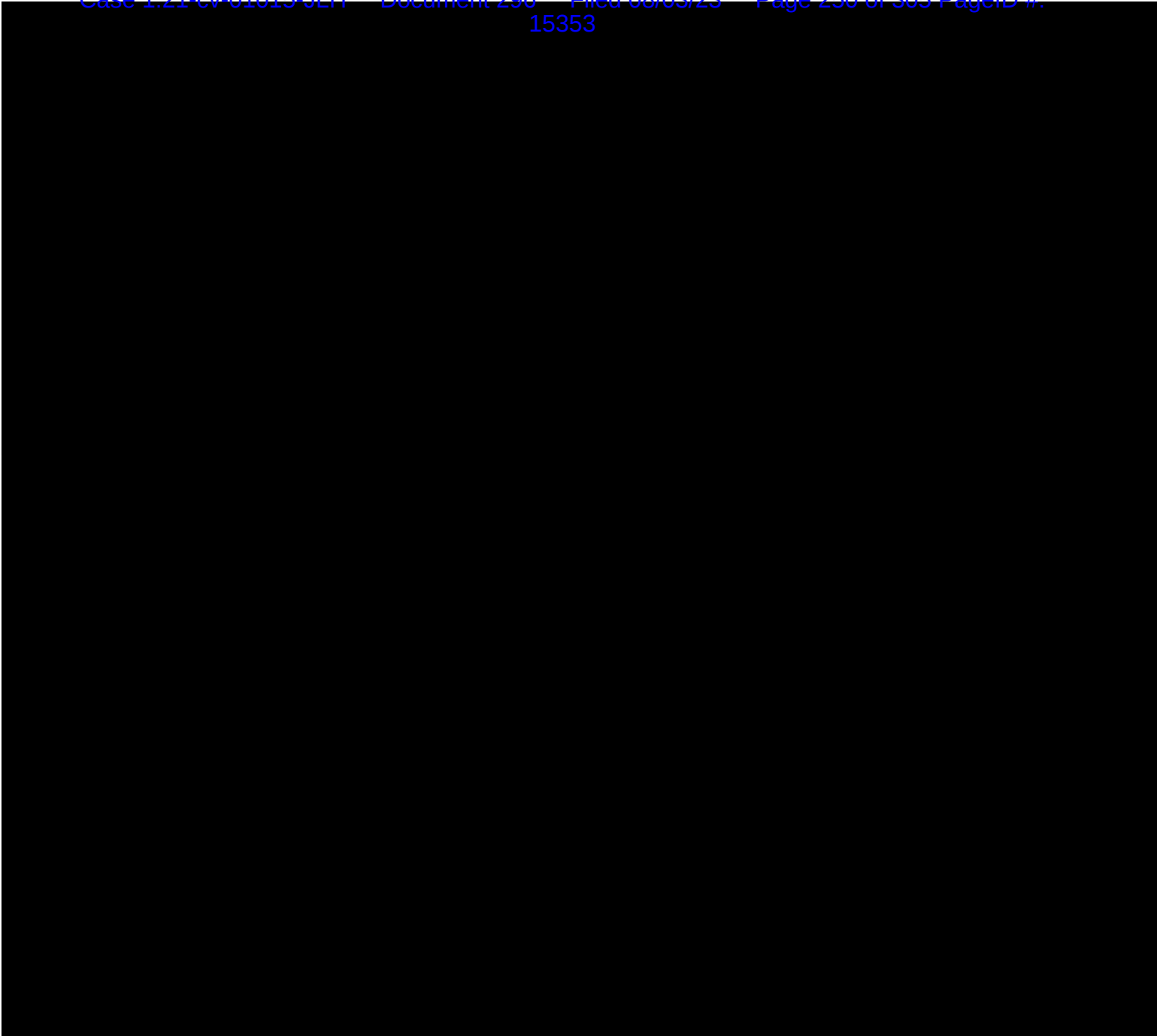


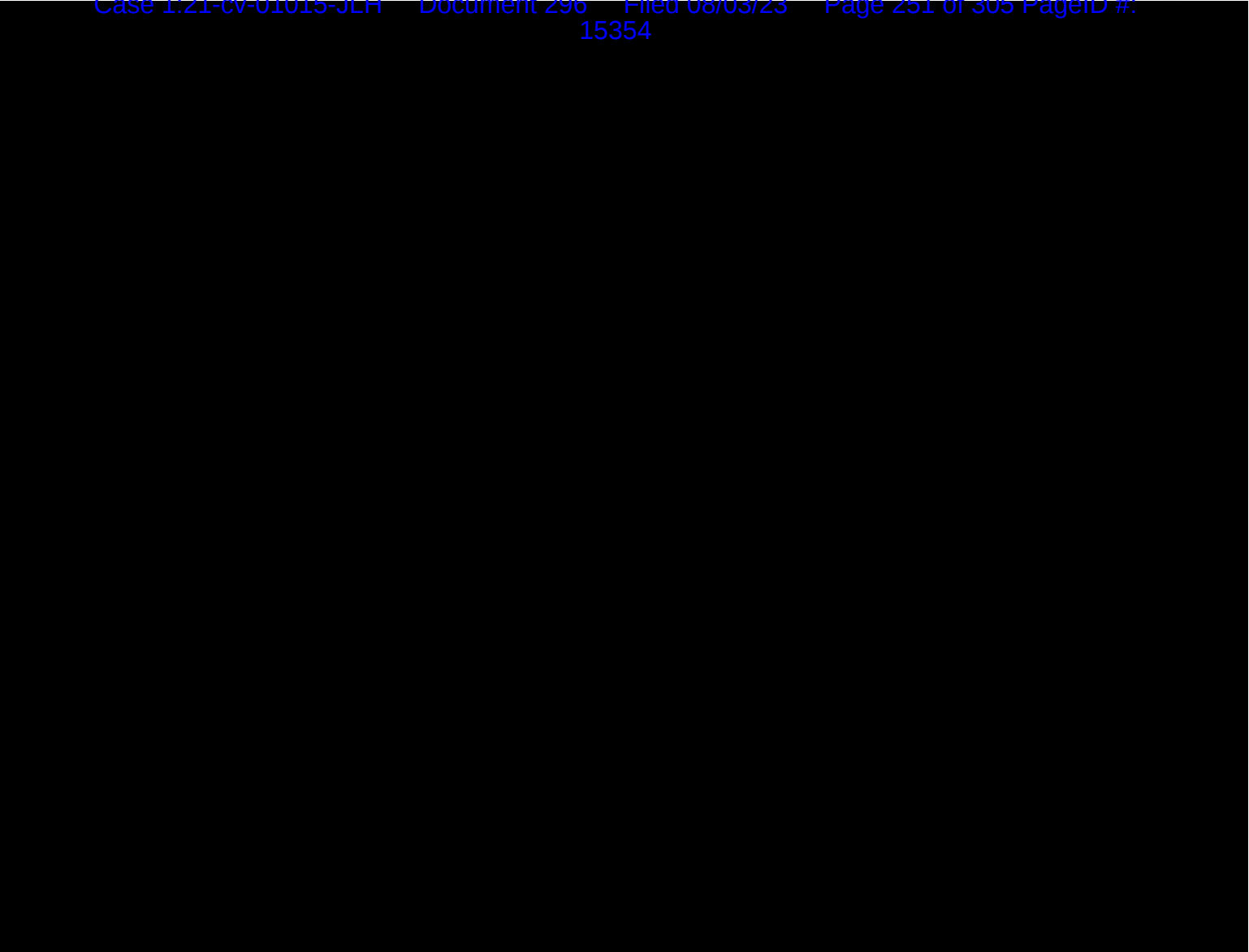


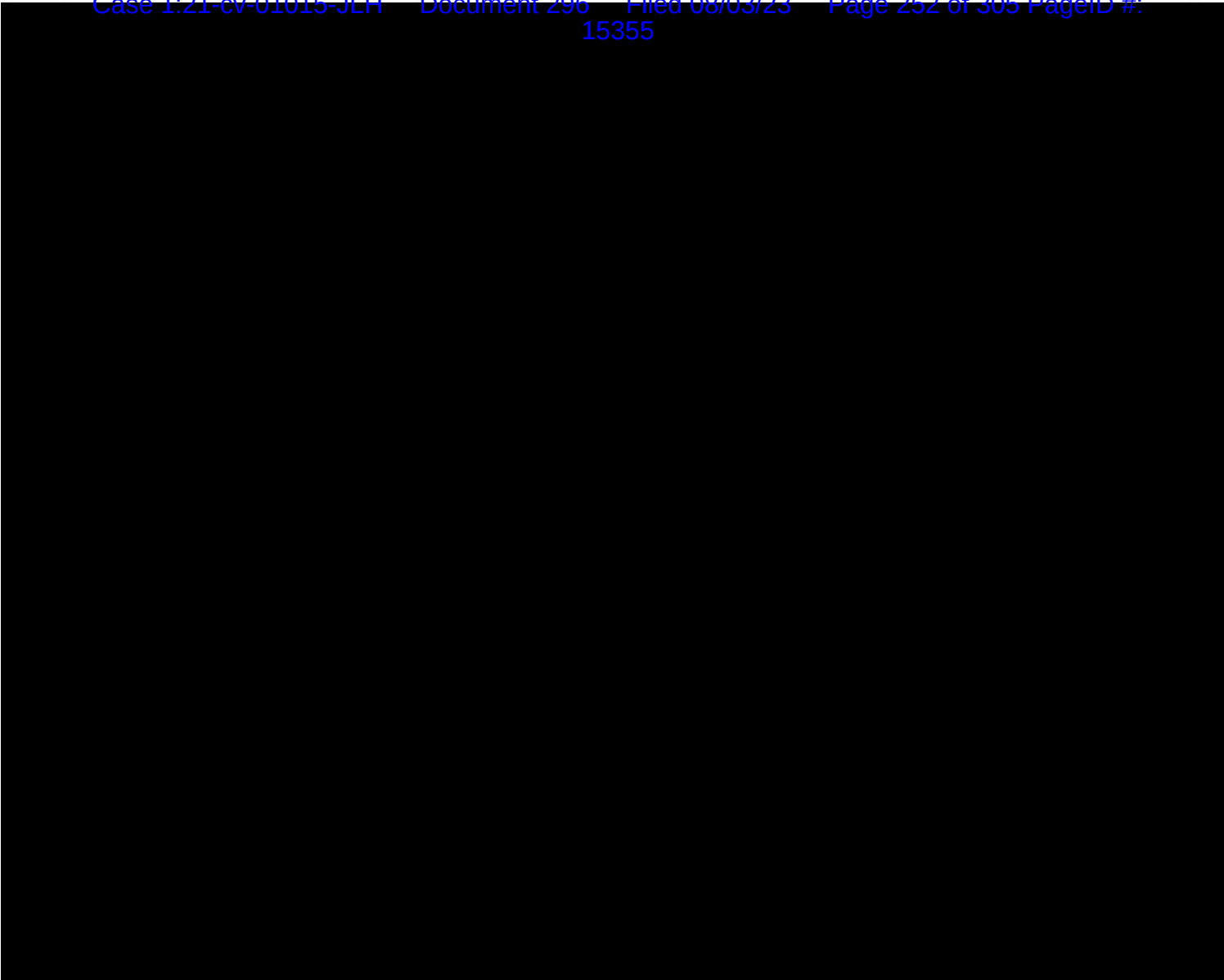




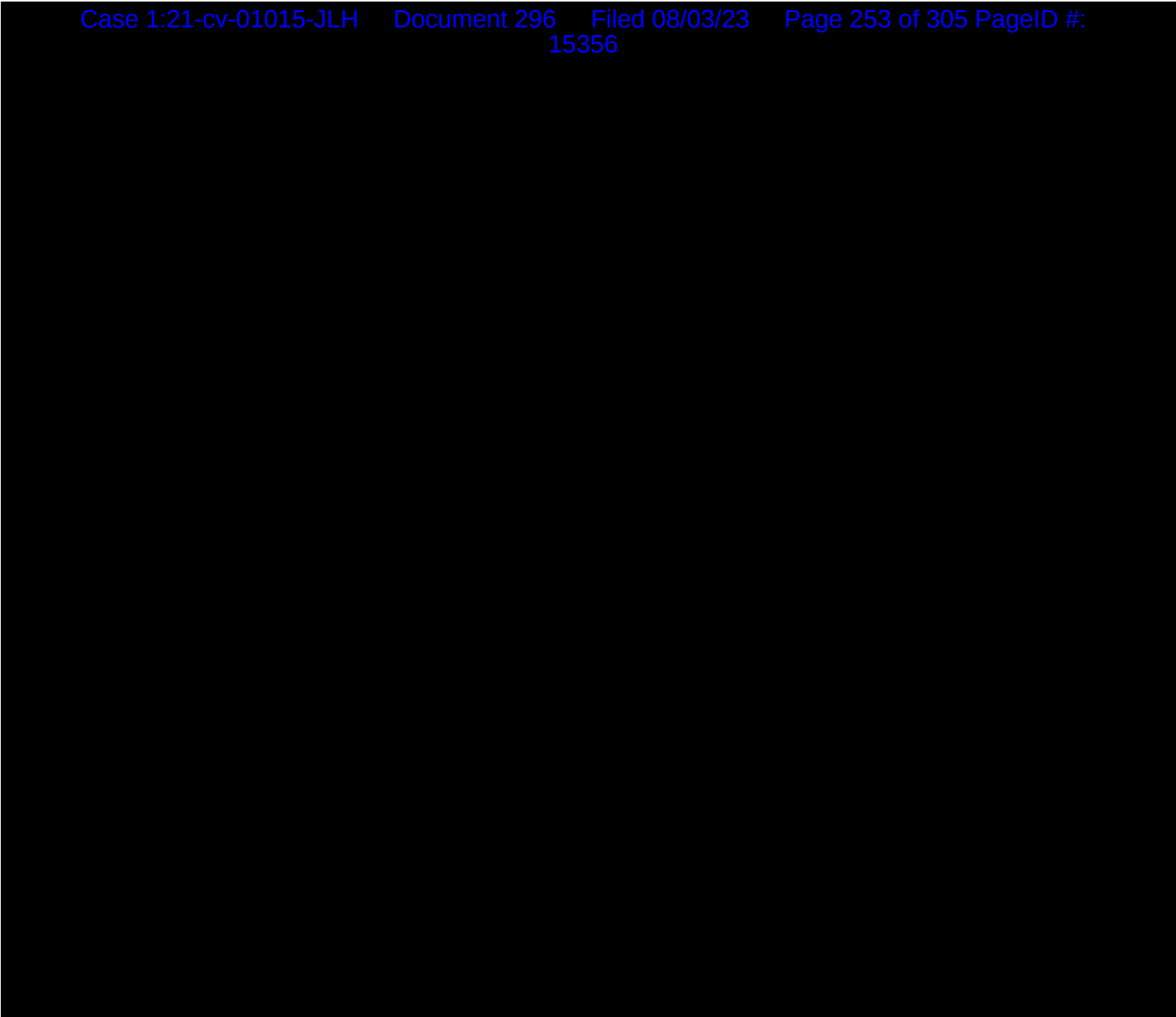


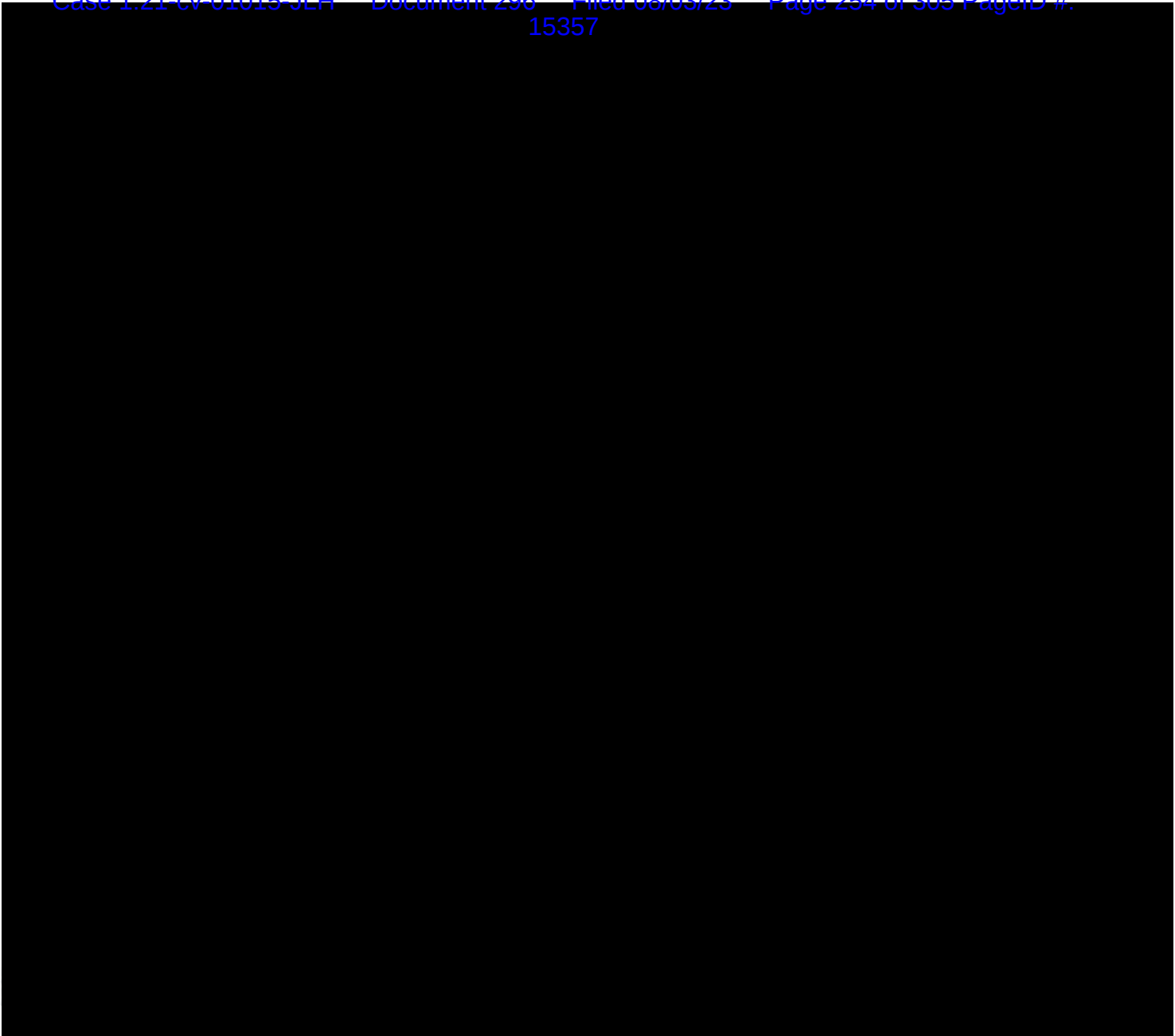


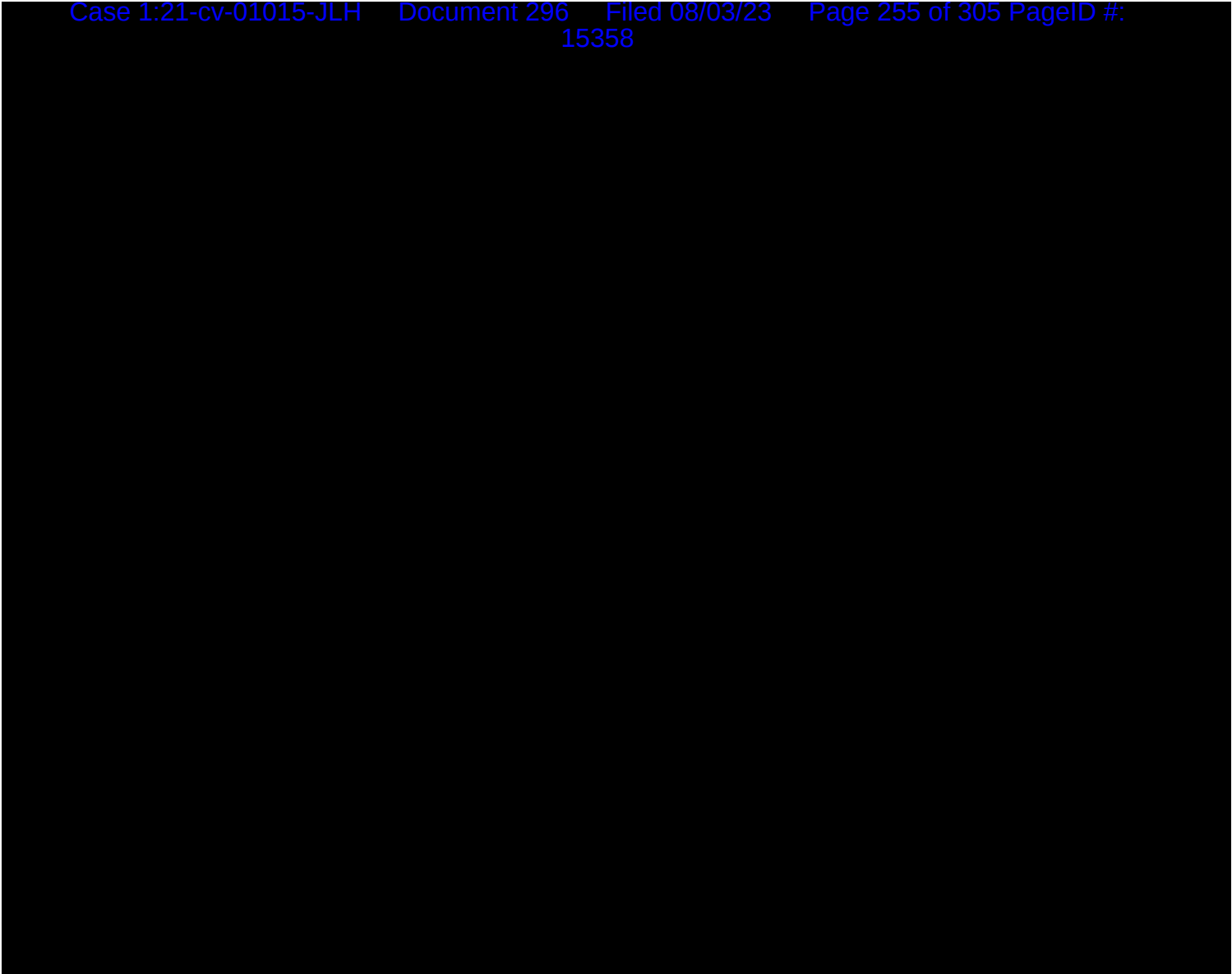


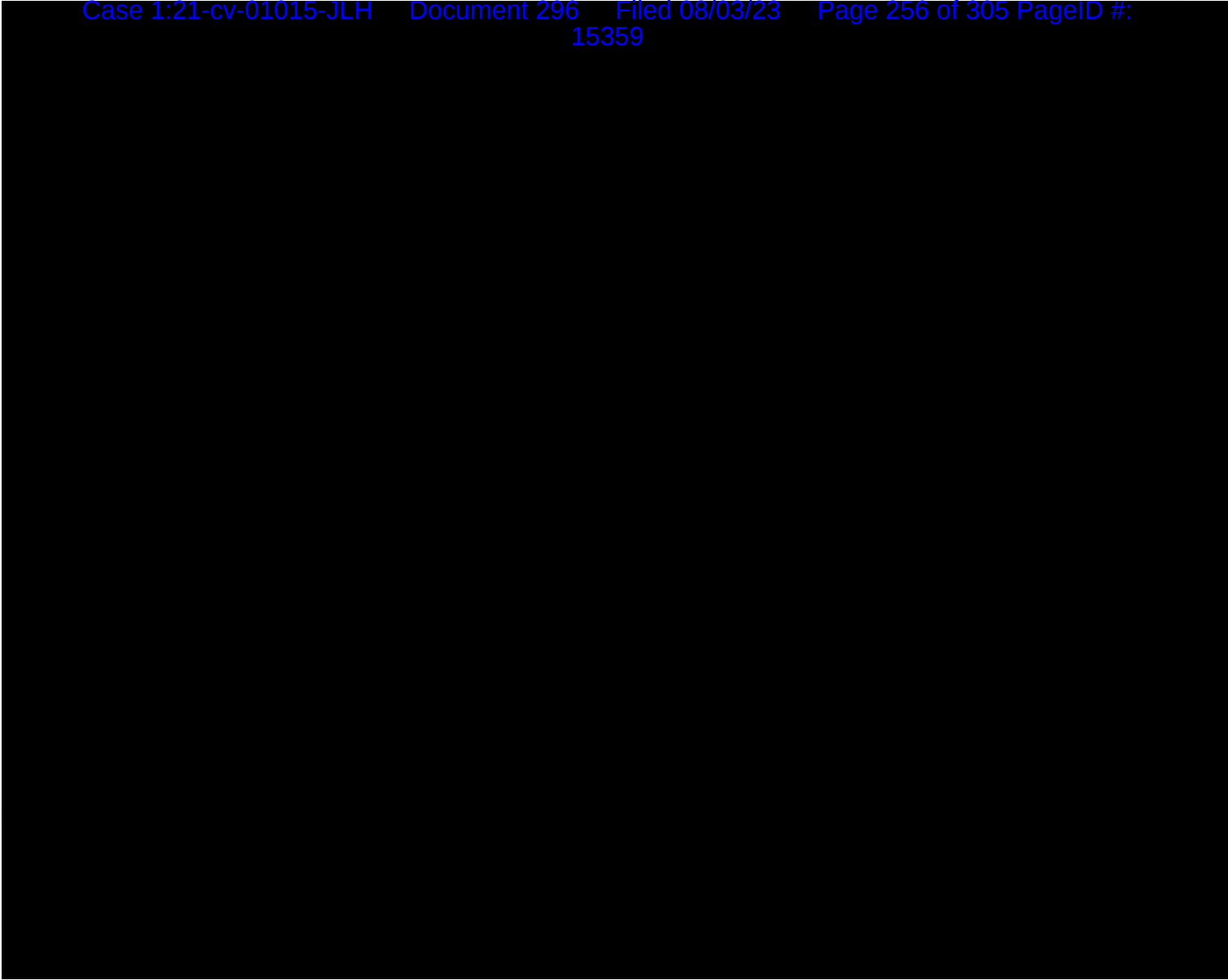




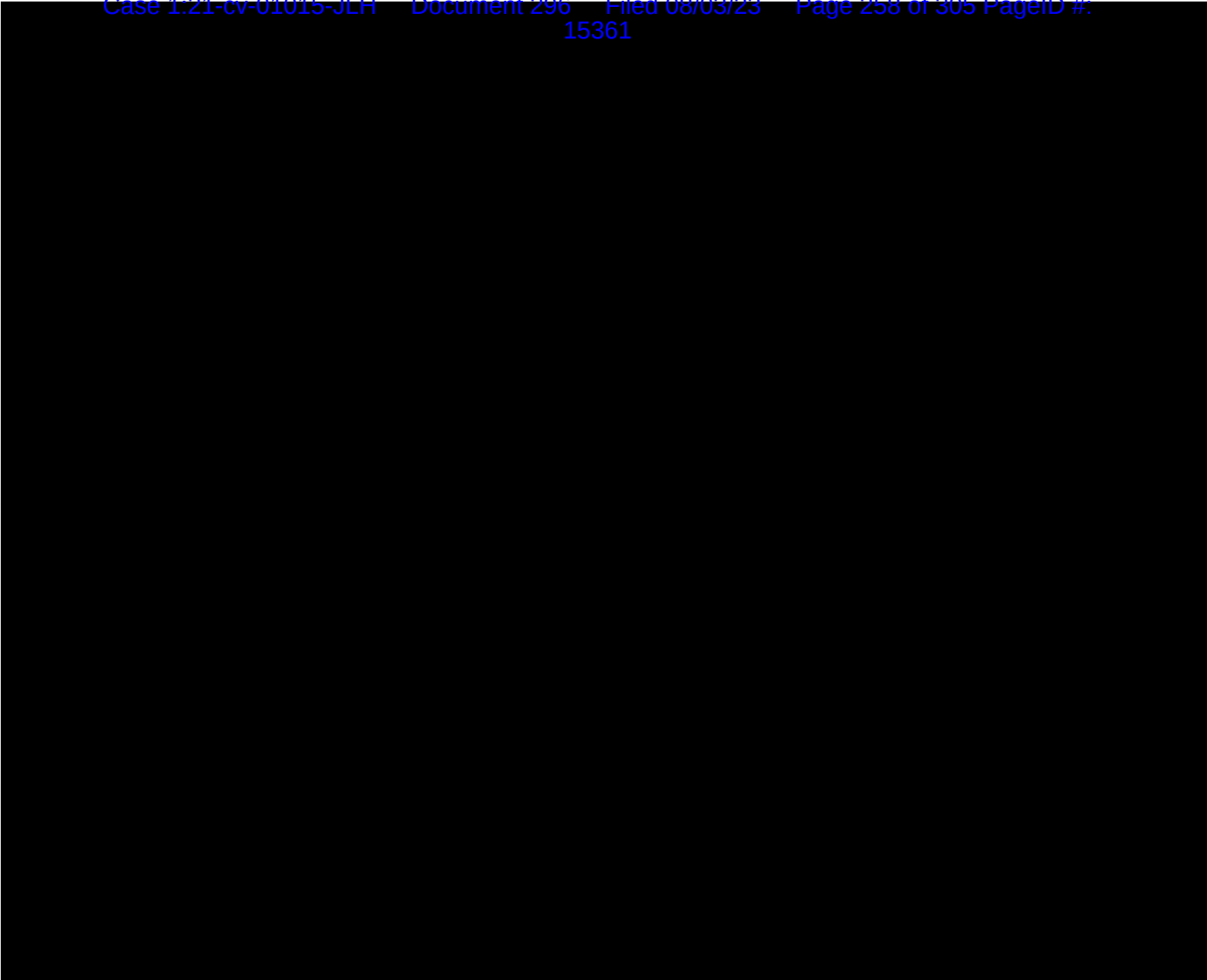


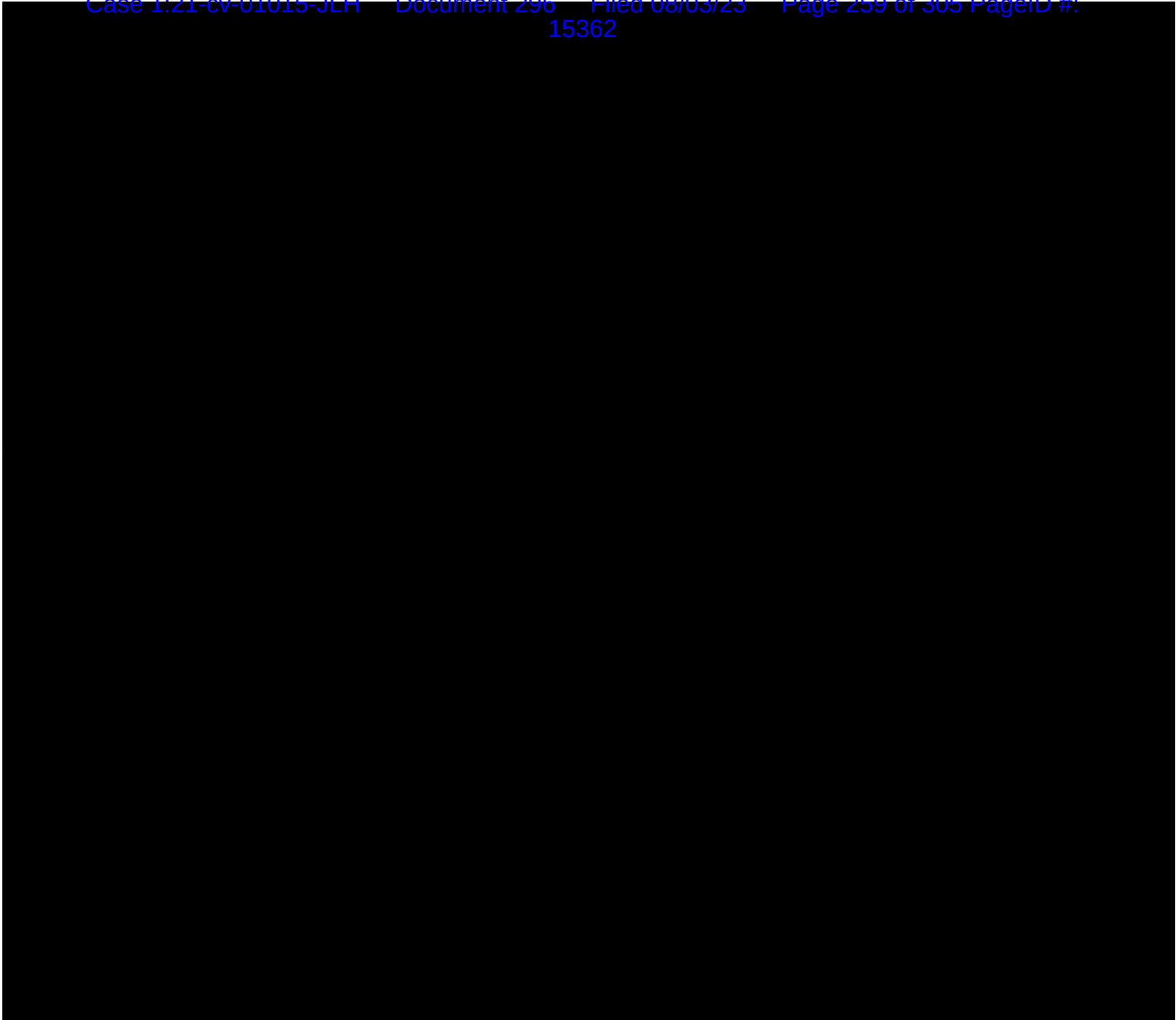


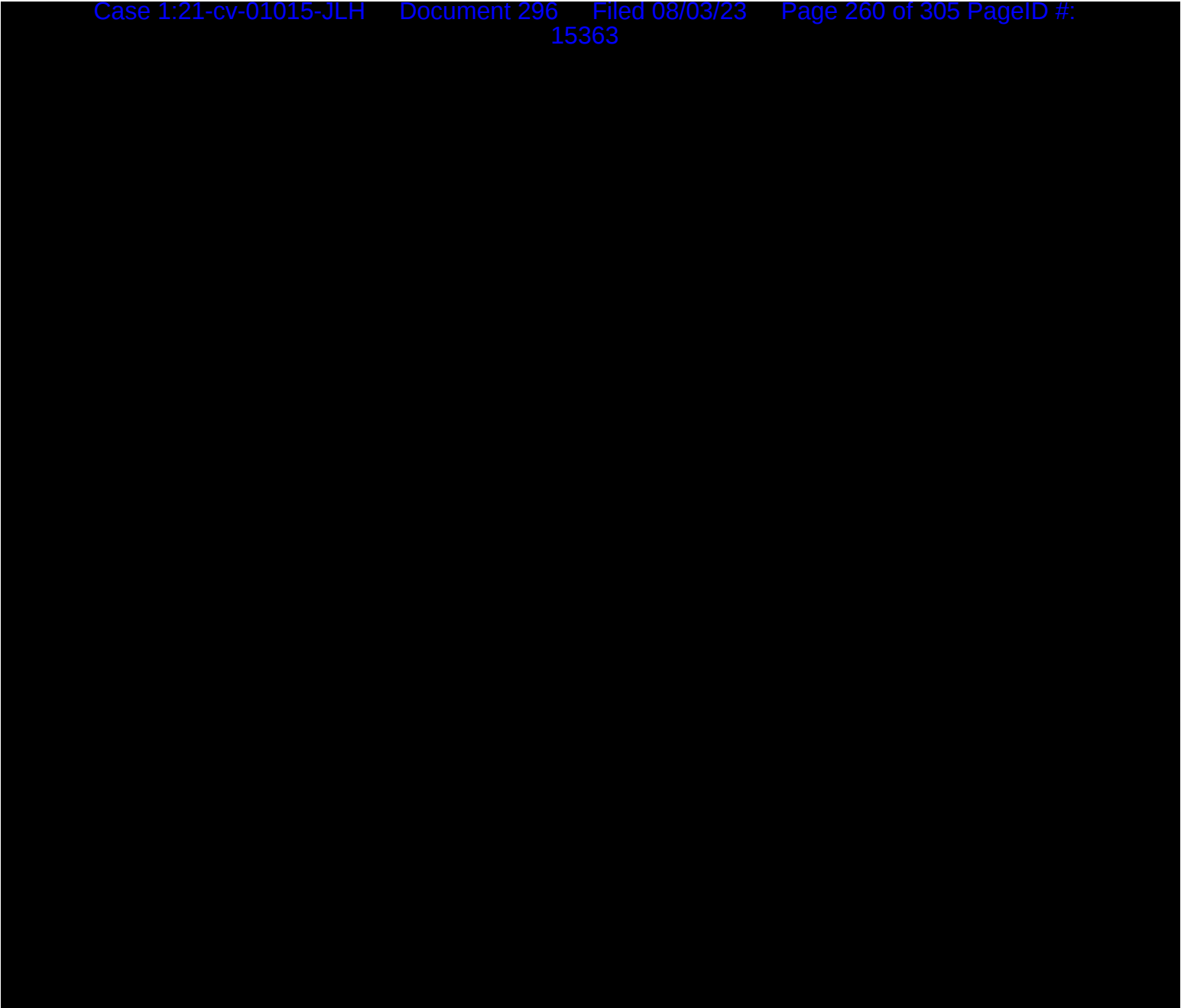




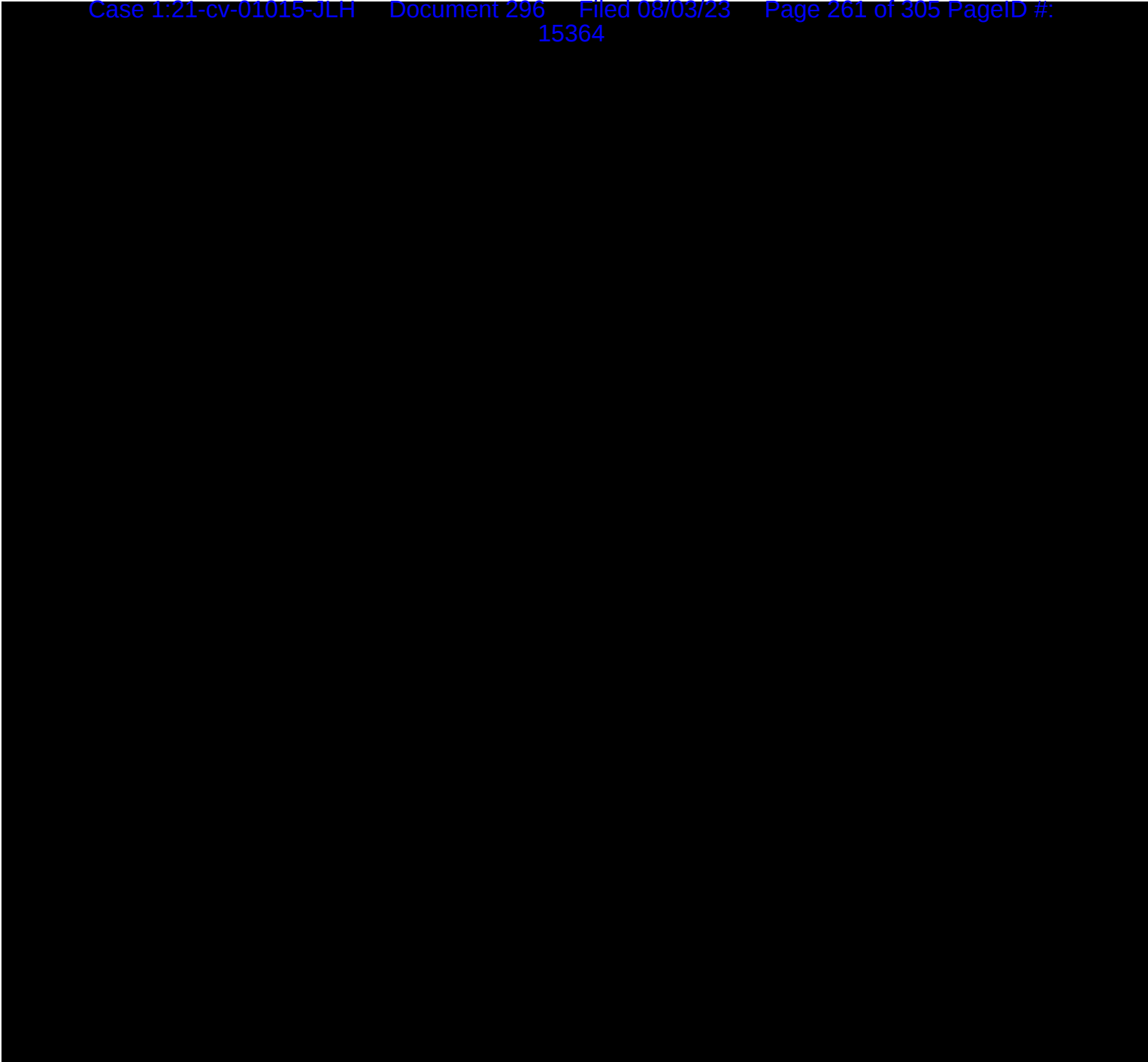


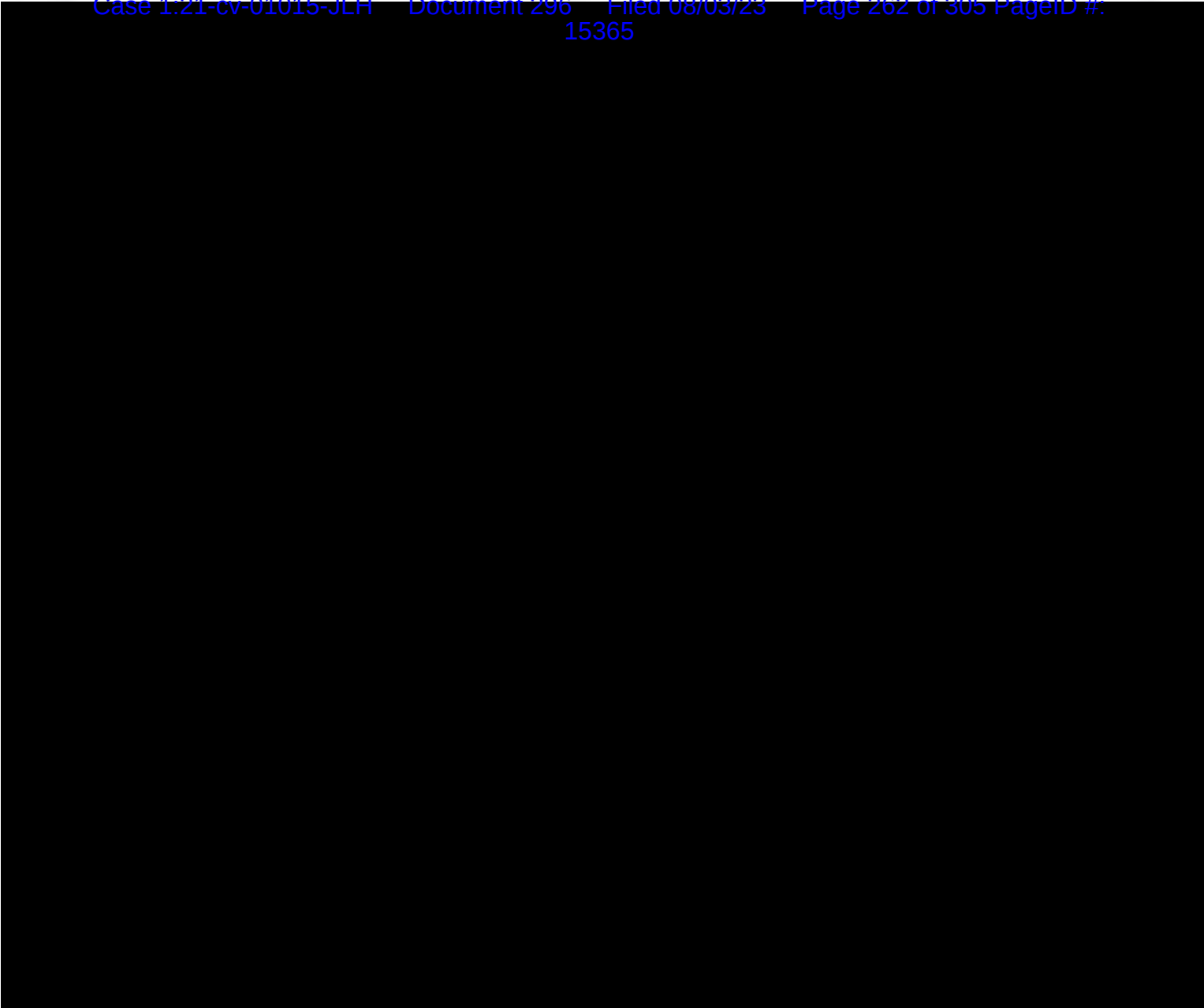


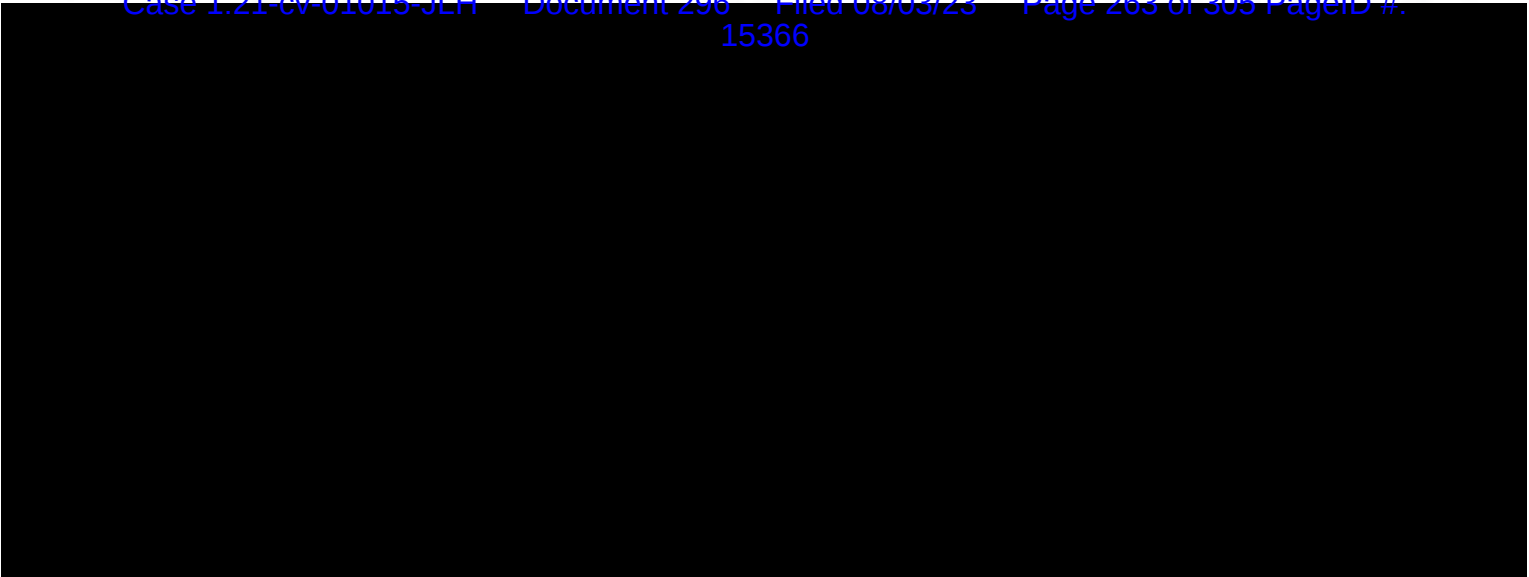












IN WITNESS WHEREOF, the Parties have executed this Agreement through their duly authorized representatives to be effective as of the Execution Date.

Sarepta Therapeutics Three, LLC

By: /s/ Peter Walsh

Name: Peter Walsh

Title: Manager

F. HOFFMANN-LA ROCHE LTD

By: /s/ James Sabky

Name: James Sabky

Title: Global Head, Pharma Partnering

By: /s/ Stefan Arnold

Name: Stefan Arnold

Title: Head Legal Pharma

79445843\_10

OUTSIDE COUNSEL EYES ONLY

PAGES: 1-230

EXHIBITS: 1-12

IN THE UNITED STATES DISTRICT COURT

DISTRICT OF DELAWARE

NO. C.A. NO. 21-1015 (GBW)

\_\_\_\_\_)  
NIPPON SHINYAKU CO., LTD., )  
Plaintiff, )  
vs. )  
SAREPTA THERAPEUTICS, INC., )  
Defendant. )  
\_\_\_\_\_)  
SAREPTA THERAPEUTICS, INC., )  
Defendant and )  
Counter-Plaintiff )  
vs. )  
NIPPON SHINYAKU CO., LTD and )  
NS PHARMA, INC., )  
Plaintiffs and )  
Counter-Defendants.)  
\_\_\_\_\_)

VIDEOTAPED DEPOSITION OF SAREPTA

THERAPEUTICS BY [REDACTED], called as a witness  
by and on behalf of Nippon Shinyaku, pursuant to  
the applicable provisions of the Federal Rules of  
Civil Procedure, Rule 30(b)(6), before P. Jodi  
Ohnemus, RPR, RMR, CRR, CA-CSR #13192, NH-LSR #91,  
MA-CSR #123193, and Notary Public, within and for  
the Commonwealth of Massachusetts, at Burns &  
Levinson, 125 High Street, Boston, Massachusetts,  
on Wednesday, June 14, 2023, commencing at 9:14  
a.m.

**APPEARANCES:**

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**For Nippon Shinyaku Co., Ltd.**

**FINNEGAN, HENDERSON, FARABOW, GARRETT**

**& DUNNER, LLP**

**BY: Ryan P. O'Quinn, Esq.**

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**Kaitlyn.pehrson@finnegan.com**

**For Sarepta Therapeutics, Inc.**

**APPEARANCES: (CONT'D)**

**ALSO PRESENT:**

**Jessica Driscoll, Esq.**

**In-House Counsel**

**Sarepta Therapeutics, Inc.**

**Adam Cerro, Video Operator**

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**I N D E X**

**TESTIMONY OF:**

**PAGE**

[REDACTED]

**(By Ms. Venegas)**

**8**



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**E X H I B I T S**

<b>EXHIBIT</b>	<b>DESCRIPTION</b>	<b>PAGE</b>
Exhibit 1	Notice to Take Depositions	12
Exhibit 2	Nippon Shinyaku Co. Ltd. and NS Pharma, Inc.'s Notice of Deposition of Sarepta Therapeutics Pursuant to Fed. R. Civ. P. 30(b)(6)	13
Exhibit 3	[REDACTED] LinkedIn profile	16
Exhibit 4	chart, two-page document	112
Exhibit 5	[REDACTED] [REDACTED], SRPT-VYDS-0210679-695	114
Exhibit 6	Excel spreadsheet, SRPT-VYDS-0210696	139
Exhibit 7	[REDACTED] [REDACTED], SRPT-VYDS-0210727-791	154
Exhibit 8	spreadsheet, SRPT-VYDS-0210792 [REDACTED],	182 188

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1	Exhibit 9	6/8/2021,	
2		SRPT-VYDS-0209787-791	
3	Exhibit 10	email, 3/9/2020,	208
4		SRPT-VYDS-0209352-353	
5	Exhibit 11	[REDACTED],	213
6		Updates as of August 26	
7		2020, SRPT-VYDS-0209514-517	
8	Exhibit 12	[REDACTED]	221
9		[REDACTED],	
10		SRPT-VYDS-0211555-623	
11			
12			
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1 VIDEO OPERATOR: Good morning. We are now  
2 on the record. The time is 9:14 a.m. Today's date  
3 is June 14th, 2023. My name is Adam Cerro. I am  
4 the videographer with AMG Reporting. This is the  
5 video deposition of [REDACTED] in the matter of  
6 Nippon Shinyaku v. Sarepta.

7 Would counsel please state their  
8 appearances, beginning with the noticing attorney.

9 MS. VENEGAS: Good morning. Krista  
10 Venegas with Morgan Lewis on behalf of Nippon  
11 Shinyaku.

12 MR. SIKORA: Mike Sikora on behalf of  
13 Nippon Shinyaku.

14 MR. O'QUINN: Ryan O'Quinn for Finnegan,  
15 Henderson on behalf of Sarepta and the University  
16 of Western Australia. With me today is my  
17 colleague Kaitlyn Pehrson and Jessica Driscoll of  
18 Sarepta Therapeutics.

19 MS. VENEGAS: Madam Court Reporter, the  
20 real-time is not following.

21 VIDEO OPERATOR: The court reporter is  
22 Jodi Ohnemus and may now swear in the witness.

23 [REDACTED], having  
24 satisfactorily been identified by  
25 the production of a driver's license,

1 and being first duly sworn by the Notary  
2 Public, was examined and testified as  
3 follows to interrogatories

4 BY MS. VENEGAS:

5 Q. Good morning. Thank you for coming to  
6 spend time with us today.

7 Can you go ahead and state your name and  
8 address for the record, please.

9 A. It's [REDACTED]. My work address is 215  
10 First Street, Cambridge, Massachusetts.

11 Q. All right. And have you ever been deposed  
12 before?

13 A. No.

14 Q. First time?

15 A. (Witness nods.)

16 COURT REPORTER: Can you say yes or no and  
17 don't nod your head, please.

18 A. Right. No.

19 Q. And the court reporter may or may not  
20 remind you. So I will. You need to go ahead and  
21 give a verbal answer, a yes or no, rather than a  
22 nod -- a nod of the head so that she can record  
23 your responses.

24 A. Okay.

25 Q. Great. So I'm going to ask you a series

1 Q. Have you seen any documents internal to  
2 Sarepta explaining [REDACTED]  
3 [REDACTED]?

4 MR. O'QUINN: Objection to form.  
5 Foundation.

6 A. [REDACTED], not that I can  
7 recall.

8 Q. So the only information that you recall  
9 seeing relating to the [REDACTED]  
10 [REDACTED]?

11 A. [REDACTED].

12 MR. O'QUINN: If we're getting ready to  
13 switch topics, Counsel, could we take a break?

14 MS. VENEGAS: Sure. Yeah, I was just  
15 going to...

16 VIDEO OPERATOR: The time is 11:28 a.m.  
17 We're off the record.

18 (Recess was taken.)

19 VIDEO OPERATOR: The time is 11:38 a.m.  
20 We're on the record.

21 Q. So before the break we were talking about  
22 [REDACTED]  
23 [REDACTED].

24 Do you recall that?

25 A. Yes.

1 Q. Okay. I'm going to shift the focus a  
2 little bit in terms of [REDACTED].  
3 We talked earlier about the gene therapy product  
4 SRP-9001.

5 Do you recall that?

6 A. Yes.



23 Q. And what do you mean by the [REDACTED]  
24 [REDACTED]? I assume you mean

25 [REDACTED]

1 [REDACTED] is that  
2 what you mean?

3 MR. O'QUINN: Objection to form.

4 A. We [REDACTED]  
5 [REDACTED]. Yeah.

6 Q. [REDACTED]  
7 [REDACTED]?

8 MR. O'QUINN: Objection to form.

9 A. We [REDACTED]  
10 [REDACTED]  
11 [REDACTED]  
12 [REDACTED].

13 Q. All right. And when you say "[REDACTED]"  
14 [REDACTED]  
15 [REDACTED].

16 Is that what you mean by "[REDACTED]"  
17 [REDACTED]?"

18 A. [REDACTED].

19 Q. And what else do you mean by "[REDACTED]"  
20 [REDACTED]?"

21 A. I mean [REDACTED].

22 Q. Okay. What is your understanding as to  
23 [REDACTED]  
24 [REDACTED]?

25 MR. O'QUINN: Objection to form. Outside

1 assumptions, I'm not -- I don't.

2 Q. I just want to confirm my understanding of  
3 one of your responses.

4 Is it correct that Sarepta [REDACTED]  
5 [REDACTED]  
6 [REDACTED]

7 [REDACTED]?

8 MR. O'QUINN: Objection to form.  
9 Foundation.

10 A. We [REDACTED]  
11 [REDACTED]  
12 [REDACTED]

13 [REDACTED].

14 MS. VENEGAS: I'm so sorry about that.

15 Q. And I was going to ask you a [REDACTED]  
16 [REDACTED]  
17 [REDACTED]  
18 [REDACTED].

19 Does Sarepta have an [REDACTED]  
20 [REDACTED]  
21 [REDACTED]  
22 [REDACTED]?

23 A. Yes. There's [REDACTED]  
24 [REDACTED].

25 Q. And what -- what are Sarepta's [REDACTED]



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1 [REDACTED]  
[REDACTED]?

3 A. [REDACTED]  
[REDACTED].

5 Q. And why not?

6 A. As I mentioned, [REDACTED]  
[REDACTED].

8 Q. And [REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED]?

12 A. I mean, if [REDACTED]  
[REDACTED].

[REDACTED]

[REDACTED]

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[REDACTED]

[REDACTED]

17 Q. I understand.

18 So, as you sit here today, for [REDACTED]

[REDACTED]

[REDACTED]

[REDACTED]

[REDACTED]

[REDACTED]

[REDACTED] ?

25 MR. O'QUINN: Objection to form. Vague.

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1 Compound.

2 A. I [REDACTED]  
[REDACTED].

4 Q. But [REDACTED]  
[REDACTED]?

6 MR. O'QUINN: Objection to form.

7 Foundation.

8 A. [REDACTED].

9 Q. Okay. So based [REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED]

[REDACTED]?

14 MR. O'QUINN: Objection to form.

15 Foundation. Vague.

16 A. I'm sorry. Can you ask that again. I  
17 just want to make sure I'm following.

18 Q. Sure.

19 [REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED]?

23 A. Yes.

24 Q. [REDACTED]  
[REDACTED].

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1 [REDACTED]  
[REDACTED].

3 Q. Okay. [REDACTED]  
[REDACTED]  
[REDACTED] -- sorry.

6 Is [REDACTED]  
[REDACTED]  
[REDACTED]?

9 MR. O'QUINN: Objection to form.

10 A. I mean, it [REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED].

16 Q. Is there [REDACTED]  
[REDACTED]  
[REDACTED]?

19 A. [REDACTED].

20 Q. I [REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED]?

24 A. [REDACTED]  
[REDACTED]

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1

[REDACTED]

[REDACTED]

[REDACTED].

4

Q. All right. Then, [REDACTED]

[REDACTED]

[REDACTED]; correct?

7

A. [REDACTED].

8

Q. Okay. [REDACTED]

[REDACTED]?

10

A. [REDACTED]

[REDACTED]

[REDACTED]"

[REDACTED]

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1 Q. All right. [REDACTED]  
[REDACTED]. So you should be able to just  
3 scroll across.

4 A. Okay.

5 Q. Okay. [REDACTED]  
[REDACTED]  
[REDACTED]; correct?

8 A. Yes.

9 Q. And [REDACTED]?

10 A. It appears -- [REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED].

16 Q. Okay. So, for example, r [REDACTED]  
[REDACTED]  
[REDACTED]; correct?

19 A. [REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED].

24 Q. Okay. [REDACTED]  
[REDACTED]

1           ROUGH DRAFT DISCLAIMER

2

3           IMPORTANT NOTICE:

4           AGREEMENT OF PARTIES

5

6                       We, the Party Working With Rough Draft  
7           Transcripts, Understand That If We Choose to Use the  
8           Rough Draft Or the Printout, That We Are Doing So With  
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IN THE UNITED STATES DISTRICT COURT  
DISTRICT OF DELAWARE

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NIPPON SHINYAKU CO., LTD.,

3

Plaintiff,

4

5

-against- C.A. No:  
21-1015(GBW)

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SAREPTA THERAPEUTICS, INC.,

8

Defendant.

9

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SAREPTA THERAPEUTICS, INC. and  
THE UNIVERSITY of WESTERN AUSTRALIA  
Defendant/Counter-Plaintiffs,

10

11

V.

12

NIPPON SHINYAKU CO. LTD. And

13

NS PHARMA. INC.,

14

Plaintiff/Counter-Defendants.

15

-----X

16

VIDEOTAPED DEPOSITION of the Defendant,

17

SAREPTA THERAPEUTICS, INC. by [REDACTED], taken by

18

the Plaintiff, pursuant to Notice, held at the law

19

offices of Finnegan, Henderson, Farabow, Garrett &

20

Dunner, LLP 2 Seaport Lane Boston Massachusetts 02210,



21 on July 25, 2023, at [!JOB START TIME], before a Notary  
22 Public of the State of New York.

23 \*\*\*\*\*  
24 HIGHLY CONFIDENTIAL-AEO\*\*\*\*  
25

3

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11

12

13

14 ALSO PRESENT:

15

GEOFFREY BASSETT-Videographer  
AMG Reporting

16 JESSICA DRISCOLL-Inhouse Counsel for Sarepta

17

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1 THE VIDEOGRAPHER: Good morning,  
2 everyone. Today's date is July 25, 2023, and  
3 the time is 9:15 a.m. Eastern Standard Time,  
4 and we are on the record. You're here today  
5 for the video-recorded deposition of [REDACTED]  
6 in the matter of Nippon Shinyaku Co., LT --  
7 Limited vs. Sarepta Therapeutics, Incorporated.  
8 Is that correct?

9 My name is Geoffrey Bassett with AMG  
10 Reporting. And the court reporter today is  
11 Brooke Perry. At this time, I will ask counsel  
12 to introduce themselves for the record.

13 MS. VENEGAS: Krista Venegas with  
14 Morgan Lewis on behalf of Nippon Shinyaku and  
15 NS Pharma.

16 MR. O'QUINN: Ryan O'Quinn with  
17 Finnegan on behalf of Sarepta Therapeutics, the  
18 University of Western Australia, and the  
19 witness.

20 THE VIDEOGRAPHER: All right. At this  
21 time, I will hand it over to the court  
22 reporter.

23 [REDACTED], the witness herein, having  
24 been first duly sworn by a Notary Public of the State of  
25 New York, was examined and testified as follows:

5

1 EXAMINATION BY

2 THE REPORTER: Please, state your name  
3 for the record.

4 THE WITNESS: [REDACTED].

5 THE REPORTER: Please, state your  
6 address for the record.

7 THE WITNESS: 215 First Street,  
8 Cambridge, Massachusetts 02142.

9 MS. VENEGAS: Good morning. And during  
10 the course of the day, Mike Sikora, also of  
11 Morgan Lewis, will be joining us. He's not  
12 present in the room at the moment, but he will

11 Q. And this [REDACTED]  
[REDACTED] ?  
13 A. Yes.  
14 Q. Okay. A [REDACTED]  
[REDACTED]  
[REDACTED], correct?  
17 A. Yes.  
18 Q. [REDACTED]  
[REDACTED] ?  
20 A. It's a [REDACTED].  
21 Q. And what is the [REDACTED]  
[REDACTED] ?  
23 MR. O'QUINN: Outside the scope.  
24 You can answer, if you know.  
25 A. [REDACTED]

207

1 [REDACTED]  
[REDACTED].  
3 Q. [REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED] ?

8 A. [REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED].

12 Q. And --

13 A. And [REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED].

17 Q. And when you say [REDACTED]  
[REDACTED]?

19 A. Yes.

20 Q. And [REDACTED]  
[REDACTED]  
[REDACTED]?

23 A. T [REDACTED]  
[REDACTED]  
[REDACTED]

208

1 Q. [REDACTED]  
[REDACTED] is that right?

3 A. Correct.

4 Q. And [REDACTED]  
[REDACTED] is that right?

6 A. [REDACTED].

7 Q. And [REDACTED]

8 [REDACTED]

9 [REDACTED]

10 [REDACTED]?

11 MR. O'QUINN: Objection to the extent it  
12 seeks a legal conclusion.

13 A. [REDACTED] [REDACTED]

14 [REDACTED]

15 [REDACTED]

16 [REDACTED]t.

17 Q. [REDACTED]?

18 A. [REDACTED]

19 [REDACTED].

20 Q. And so [REDACTED]

21 [REDACTED]

22 [REDACTED]?

23 A. [REDACTED]

24 Q. But the [REDACTED]

25 [REDACTED]

209

1 correct?

2 A. [REDACTED]

3 Q. And as you sit here today, you [REDACTED]  
[REDACTED], correct?

5 MR. O'QUINN: Objection to the extent it  
6 it seeks a legal conclusion. Outside the  
7 scope. You can answer if you know.

8 Q. Let's [REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED], correct.

13 A. [REDACTED]

14 Q. And if you look [REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED], correct?

19 MR. O'QUINN: Objection to the extent  
20 it seeks a legal conclusion. You can answer.

21 A. [REDACTED]  
[REDACTED]

23 Q. Right. And as we discussed earlier, [REDACTED]  
[REDACTED] correct?

25 A. [REDACTED]

1 [REDACTED] --

2 Q. Under [REDACTED]

3 [REDACTED]

4 [REDACTED], correct?

5 A. C [REDACTED]

6 [REDACTED]

7 [REDACTED]

8 [REDACTED]

9 [REDACTED]

10 [REDACTED]

11 [REDACTED]

12 [REDACTED].

13 Q. Right. So let's turn to [REDACTED]

14 [REDACTED]

15 [REDACTED]

16 [REDACTED]

17 [REDACTED]?

18 A. [REDACTED]

19 [REDACTED]

20 [REDACTED]

21 [REDACTED]

22 [REDACTED] --

23 Q. [REDACTED]

24 [REDACTED]

25 [REDACTED]



1 [REDACTED], correct?

2 MR. O'QUINN: Object to the extent it  
3 seeks a legal conclusion and calls for  
4 speculation. You can answer.

5 A. [REDACTED]

6 [REDACTED]

7 [REDACTED]

8 [REDACTED]

9 [REDACTED]

10 Q. [REDACTED]

11 [REDACTED]

12 [REDACTED], correct?

13 A. [REDACTED].

14 Q. [REDACTED]

15 [REDACTED]

16 [REDACTED] correct?

17 MR. O'QUINN: Objection to form.

18 Foundation. Calls for a conclusion.

19 A. [REDACTED]

20 [REDACTED]

21 [REDACTED].

22 Q. [REDACTED]

23 [REDACTED],

24 correct?

25 A. [REDACTED]

212

1 Q. [REDACTED]

[REDACTED]

[REDACTED], correct?

4 MR. O'QUINN: Objection to form.

5 Foundation to the extent it calls for a legal

6 conclusion.

7 A. [REDACTED]

[REDACTED]

9 Q. [REDACTED]

[REDACTED]?

11 MR. O'QUINN: Objection to form.

12 Foundation.

13 A. [REDACTED]

14 Q. A [REDACTED]

[REDACTED]

[REDACTED]

[REDACTED]

18 Do you recall that?

19 MR. O'QUINN: Objection to the extent it

20 misrepresents the document.

21 A. [REDACTED]

22 Q. [REDACTED]

[REDACTED]

[REDACTED]

25 A. [REDACTED]

213

1 [REDACTED]?

2 Q. [REDACTED]

3 A. [REDACTED]

4 Q. [REDACTED]

5 [REDACTED]

6 [REDACTED]

7 A. [REDACTED]

8 Q. [REDACTED]

9 [REDACTED]

10 A. [REDACTED]

11 Q. [REDACTED]

[REDACTED], correct?

13 A. [REDACTED].

14 Q. [REDACTED]

[REDACTED]

[REDACTED]

[REDACTED]

18 [REDACTED], right?

19 A. [REDACTED]

[REDACTED].

21 Q. [REDACTED]

[REDACTED]

[REDACTED]

[REDACTED]

[REDACTED].

214

1 A. [REDACTED].

2 MS. VENEGAS: You can put those  
3 agreements aside. I'm going to introduce the  
4 next Exhibit in order, which I believe is 16.

5 (Whereupon, SRPT-VYDS-0222226-64 was  
6 marked as Exhibit 16, for identification, as of  
7 this date.)

8 THE WITNESS: Are we done with 15 for  
9 now?

10 MS. VENEGAS: Correct. You can set  
11 that aside. So I'm going to introduce the next  
12 Exhibit in order, which is Exhibit 16, an  
13 e-mail with its attachment, beginning at Bates  
14 number SRPT-VYDS-0222226.

15 BY MS. VENEGAS:

16 Q. [REDACTED], have you seen this e-mail or e-mail  
17 chain and attachment before?

18 A. [REDACTED] a --

19 Q. [REDACTED]

[REDACTED] [REDACTED]

[REDACTED] [REDACTED]

[REDACTED] [REDACTED]

[REDACTED] [REDACTED]?

24 A. [REDACTED]

25 Q. [REDACTED]?

215

1 A. [REDACTED]

[REDACTED] [REDACTED].

3 Q. [REDACTED]

[REDACTED] [REDACTED]

[REDACTED] [REDACTED], correct?

6 A. [REDACTED]

7 Q. [REDACTED]?

8 A. [REDACTED].

9 Q. [REDACTED]

[REDACTED] [REDACTED]

[REDACTED] [REDACTED]

[REDACTED] [REDACTED]?

13 A. [REDACTED].

14 Q. A [REDACTED]

[REDACTED] [REDACTED]

[REDACTED] [REDACTED], correct?

17 A. [REDACTED].

18 Q. [REDACTED]

[REDACTED] [REDACTED]?

20 A. [REDACTED]

[REDACTED] [REDACTED]

[REDACTED] [REDACTED]

23 Q. [REDACTED]

[REDACTED] [REDACTED]

25 A. [REDACTED]

216

1 Q. [REDACTED]

[REDACTED] [REDACTED]?

3 A. [REDACTED]

[REDACTED] [REDACTED].

5 Q. [REDACTED]

[REDACTED] [REDACTED]

[REDACTED] [REDACTED]

8 A. [REDACTED].

9 Q. [REDACTED]

[REDACTED] [REDACTED]

11 [REDACTED]?

12 A. [REDACTED]

[REDACTED]

[REDACTED]

15 Q. [REDACTED]

[REDACTED]

[REDACTED]

[REDACTED]?

19 MR. O'QUINN: Objection to form.

20 Foundation.

21 A. [REDACTED] 1.

22 Q. [REDACTED]

[REDACTED]

[REDACTED]

[REDACTED]

217

1 [REDACTED], " right?

2 A. [REDACTED]

3 Q. [REDACTED]

[REDACTED]

[REDACTED]?

6 MR. O'QUINN: Outside the scope.

7 You can answer if you know in your

8 personal capacity.

9 A. [REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED].

14 Q. [REDACTED]  
[REDACTED]  
[REDACTED]  
[REDACTED]?

18 MR. O'QUINN: Outside the scope.

19 You can answer if you know.

20 A. [REDACTED]

21 Q. [REDACTED]  
[REDACTED]  
[REDACTED] is that right?

24 A. [REDACTED]  
[REDACTED]

218

1 [REDACTED]  
[REDACTED]  
[REDACTED].

4 Q. [REDACTED]  
[REDACTED]



6 [REDACTED]

7 A. [REDACTED]

[REDACTED] [REDACTED]

[REDACTED] [REDACTED]

[REDACTED] [REDACTED]

[REDACTED] [REDACTED]

[REDACTED] [REDACTED]

[REDACTED] [REDACTED]

[REDACTED] [REDACTED]

[REDACTED] [REDACTED]

[REDACTED] [REDACTED]

[REDACTED] [REDACTED]

[REDACTED] [REDACTED]

[REDACTED] [REDACTED]

[REDACTED] [REDACTED]

[REDACTED] [REDACTED]

[REDACTED] [REDACTED]

[REDACTED] [REDACTED]

[REDACTED] [REDACTED]

[REDACTED] [REDACTED].

219

1 Q. [REDACTED]

[REDACTED] [REDACTED]



# On an urgent mission, in partnership with patients and their families

## Disease Areas

At Sarepta, we're committed to pursuing some of the world's most debilitating, prevalent, and complex rare genetic diseases. Today, our primary focus is on Duchenne muscular dystrophy, limb-girdle muscular dystrophies, and Charcot-Marie-Tooth disease.

### Learn About Some of the Genetic Diseases We're Researching



**Duchenne muscular dystrophy**



**Limb-girdle muscular dystrophy**



Charcot-Marie-Tooth disease type 1A

## Patient Support



SareptAssist is our patient support program designed to provide you with information to help you navigate the process of starting and staying on therapy. This program is available to patients in the United States.

[Find out more](#)

# Sarepta's Patient Affairs Team



With our deep focus on supporting rare disease communities and commitment to patient centricity, Sarepta's Patient Affairs team is the bridge between our company and the patient and caregiver communities we serve. Our direct work with individuals living with rare disease supports the ethos of Sarepta by establishing a sense of urgency and drive towards action.

**Meet our team**



## Corporate Grants and Giving

Serving the rare disease community goes beyond developing precision genetic medicines. Corporate Giving is a core part of Sarepta's mission.

[Find out more](#)



## Route 79, The Duchenne Scholarship Program

Route 79, The Duchenne Scholarship Program, was created to support the post-secondary educational goals of students living with Duchenne.

[Find out more](#)

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